UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____to_____

Commission file number: 001-39438

Disc Medicine, Inc.

(Exact name of registrant as specified in its charter)

Delaware		**85-1612845**
(State or other jurisdiction of incorporation or organization)		**(I.R.S. Employer Identification No.)**
321 Arsenal Street, Suite 101 Watertown, Massachusetts		**02472**
(Address of principal executive offices)		**(Zip Code)**

Registrant's telephone number, including area code: (617) 674-9274

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.0001 Par Value	**IRON**	**The Nasdaq Global Market**

Securities registered pursuant to Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☒ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of Common Stock held by non-affiliates of the registrant computed by reference to the price of the registrant's Common Stock as of June 30, 2024, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $983.0 million (based on the last reported sale price on the Nasdaq Global Market as of such date).

As of February 21, 2025, there were 34,569,042 shares of the registrant's Common Stock, $0.0001 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for its 2025 Annual Meeting of Stockholders, which the registrant intends to file with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2024, are incorporated by reference into Part III of this Annual Report on Form 10-K.

Disc Medicine, Inc.
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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K of Disc Medicine, Inc., or the Company, contains or incorporates statements that constitute forward-looking statements within the meaning of the federal securities laws. Our forward-looking statements include, but are not limited to, statements regarding our or our management team's expectations, hopes, beliefs, intentions or strategies regarding the future. In addition, any statements that refer to projections, forecasts or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking statements. The words "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intends," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "will," "would" and similar expressions may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking. Forward-looking statements in this Annual Report on Form 10-K may include, for example, statements about:

- the initiation, timing, progress, results, and cost of our research and development programs and our current and future preclinical studies and clinical trials, including our planned APOLLO clinical trial of bitopertin, our ongoing and planned clinical trials of DISC-0974 and our planned Phase 2 clinical trial of DISC-3405, and including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available, and our research and development programs;
- our ability to efficiently discover and develop product candidates;
- our ability and the potential to successfully manufacture our drug substances and product candidates for preclinical use, for clinical trials and on a larger scale for commercial use, if approved;
- our ability to obtain funding for our operations necessary to complete further development and commercialization of our product candidates;
- our ability to seek and obtain approval for bitopertin under the U.S. Food and Drug Administration's, or FDA's, Accelerated Approval Program, including the FDA's acceptance of the APOLLO clinical study as a post-marketing confirmatory trial, the timeline for a potential New Drug Application, or NDA, submission, and whether the NDA submission will meet the standards for accelerated approval;
- our ability to obtain and maintain regulatory approval of our product candidates;
- our ability to commercialize our products, if approved;
- the pricing and reimbursement of our product candidates, if approved;
- the implementation of our business model, and strategic plans for our business and product candidates;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- estimates of our future expenses, revenues, capital requirements, and our needs for additional financing;
- the potential benefits of strategic collaboration agreements, our ability to enter into strategic collaborations or arrangements, and our ability to attract collaborators with development, regulatory and commercialization expertise;
- future agreements with third parties in connection with the commercialization of product candidates and any other approved product;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- our financial performance;
- our ability to remediate the material weakness in our internal control over financial reporting;
- the rate and degree of market acceptance of our product candidates;
- regulatory developments in the United States and foreign countries;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- our ability to produce our products or product candidates with advantages in turnaround times or manufacturing cost;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- the impact of laws and regulations;

- developments relating to our competitors and our industry;
- the impact of global economic and political developments on our business, including rising inflation and capital market disruptions, geopolitical conflicts, including the conflicts between Russia and Ukraine and in the Middle East, economic sanctions and economic slowdowns or recessions that may result from such developments which could harm our research and development efforts as well as the value of our common stock and our ability to access capital markets; and
- other risks and uncertainties, including those listed under the caption "Risk Factors."

These forward-looking statements are based on information available to us at the time of this Annual Report on Form 10-K and current expectations, forecasts and assumptions, and involve a number of judgments, risks and uncertainties. Accordingly, forward-looking statements should not be relied upon as representing our views as of any subsequent date, and we do not undertake any obligation to update forward-looking statements to reflect events or circumstances after the date they were made, whether as a result of new information, future events or otherwise, except as may be required under applicable securities laws.

The outcome of the events described in these forward-looking statements is subject to known and unknown risks, uncertainties, and other factors. As a result of a number of known and unknown risks and uncertainties, our actual results or performance may be materially different from those expressed or implied by these forward-looking statements. Factors that could cause actual results to differ include, but are not limited to, those discussed in the section titled "Risk Factors" included within Item 1A of this Annual Report on Form 10-K.

RISK FACTOR SUMMARY

Below is a summary of the principal factors that make an investment in our common stock speculative or risky. This summary does not address all of the risks that we face. Additional discussion of the risks summarized in this risk factor summary and other risks that we believe are material to our investors can be found below under the heading "Item 1A. Risk Factors" and should be carefully considered, together with other information in this Annual Report on Form 10-K and our other filings with the Securities and Exchange Commission, or SEC, before making an investment decision regarding our common stock.

- Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- We have incurred significant net losses since our inception and anticipate that we will continue to incur losses for the foreseeable future.
- We have no products approved for commercial sale and have not generated any revenue from product sales.
- Our existing and any future indebtedness could adversely affect our ability to operate our business.
- We may need to raise substantial additional funding. If we are unable to raise capital when needed or on terms acceptable to us, we may be forced to delay, reduce, or eliminate some of our product development programs or commercialization efforts.
- We have not yet progressed any product candidates into a Phase 3 clinical trial and may be unable to successfully complete any additional clinical trials for any product candidates we develop. Certain of our programs are still in preclinical development and may never advance to clinical development.
- Our programs are focused on the development of therapeutics for patients with hematologic diseases, which is a rapidly evolving area of science, and the approach we are taking to discover and develop product candidates is novel and may never lead to approved or marketable products.
- Interim, top-line, initial and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to confirmation, audit, and verification procedures that could result in material changes in the final data.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.
- Results from early preclinical studies and clinical trials of our programs and product candidates are not necessarily predictive of the results of later preclinical studies and clinical trials of our programs and product candidates. If we cannot replicate the results from earlier preclinical studies and clinical trials of our programs and product candidates in our later preclinical studies and clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.
- Our clinical trials or those of our future collaborators may reveal significant adverse events not seen in prior preclinical studies or clinical trials and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.
- Some of our product candidates modulate pathways for which there are currently no approved or effective therapies, which may result in greater research and development expenses, regulatory issues that could delay or prevent approval, or discovery of unknown or unanticipated adverse effects on safety or efficacy.
- We have conducted and are currently conducting clinical trials for bitopertin in Australia and may in the future conduct additional clinical trials of our product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.
- If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for any of our product candidates, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired.
- We face substantial competition, which may result in others discovering, developing, or commercializing products before or more successfully than we do.
- If our current product candidates or any future product candidates do not achieve broad market acceptance, the revenue that we generate from our sales may be limited, and we may never become profitable.

- We rely on third parties to conduct our clinical trials for our product candidates, as well as potential investigator-sponsored clinical trials of our product candidates. If these third parties do not successfully carry out their contractual duties, comply with regulatory requirements, or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.
- We might not realize the anticipated benefits of our current collaborations with Mabwell or NIH, or any other collaborations we enter into in the future.
- We contract with third parties for the manufacture of our product candidates for preclinical development and clinical testing, and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.
- If we are unable to obtain and maintain patent and other intellectual property protection for our technology and product candidates, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology and drugs may be impaired, and we may not be able to compete effectively in our market.
- We may not obtain or grant licenses or sublicenses to intellectual property rights in all markets on equally or sufficiently favorable terms with third parties.
- Intellectual property rights do not guarantee commercial success of current or future product candidates or other business activities. Numerous factors may limit any potential competitive advantage provided by our intellectual property rights.
- Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.
- We may seek approval from the FDA for bitopertin or any of our other current or future product candidates under the Accelerated Approval Program. If we are not able to use such program, we may be required to conduct additional clinical trials beyond those that are contemplated, which would increase the expense of obtaining, and delay the receipt of, necessary marketing approvals, if we receive them at all. In addition, even if the Accelerated Approval Program is available to us, it may not ultimately lead to expedited approval of our product candidates, or approval at all.
- Inadequate funding for the NIH, CMS, FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, including significant leadership, personnel, or policy changes, could prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.
- Our future success depends on our ability to retain key executives and experienced scientists and to attract, retain, and motivate qualified personnel.
- Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults, or non-performance by financial institutions or transactional counterparties, could adversely affect the Company's current and projected business operations and its financial condition and results of operations.
- Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.
- The market price of our common stock is expected to be volatile.
- We have incurred and will continue to incur increased costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies.
- We have identified a material weakness in our internal controls over financial reporting and may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, which may result in material misstatements of our consolidated financial statements or cause us to fail to meet our periodic reporting obligations.

This section contains forward-looking statements. You should refer to the explanation of the qualifications and limitations on forward-looking statements beginning on page ii.

PART I

Except where the context otherwise requires or where otherwise indicated, the terms "Disc," "we," "us," "our," "our company," "the company," and "our business" refer to Disc Medicine, Inc. and its consolidated subsidiaries.

ITEM 1. BUSINESS

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development, and commercialization of novel treatments for patients suffering from serious hematologic diseases. We have assembled a portfolio of clinical and preclinical product candidates that aim to modify fundamental biological pathways associated with the formation and function of red blood cells, specifically heme biosynthesis and iron homeostasis. Our current pipeline includes bitopertin for the treatment of erythropoietic porphyrias, or EPs, including erythropoietic protoporphyria, or EPP, and X-linked protoporphyria, or XLP, and Diamond-Blackfan Anemia, or DBA; DISC-0974 for the treatment of anemia of myelofibrosis, or MF, and anemia of chronic kidney disease, or CKD; and DISC-3405 (formerly MWTX-003) for the treatment of polycythemia vera, or PV, and other hematologic disorders. In addition, our preclinical programs also include DISC-0998 for the treatment of anemia associated with inflammatory diseases. Our approach to product candidate development leverages well-understood molecular mechanisms that have been validated in humans. We believe that each of our product candidates, if approved, has the potential to improve the lives of patients suffering from hematologic diseases.

Bitopertin is the lead product candidate in our heme biosynthesis modulation portfolio. Bitopertin was previously evaluated by F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., or collectively, Roche, in a comprehensive clinical program in over 4,000 individuals in other indications which demonstrated the activity of bitopertin as a glycine transporter 1, or GlyT1, inhibitor and its effect on heme biosynthesis. We are initially developing bitopertin for the treatment of EPs, including EPP and XLP, which are part of a group of severe diseases, known as porphyrias, caused by defects in the heme biosynthesis pathway that cause an accumulation of toxic metabolites referred to as porphyrins, resulting in skin hypersensitivity to sunlight and some types of artificial light. In June and December 2023, we presented interim data from BEACON, a Phase 2 open-label, parallel-dose clinical trial of bitopertin in EPP and XLP patients conducted at sites in Australia. In April 2024, we presented topline data from AURORA, a Phase 2, randomized, double-blind, placebo-controlled clinical trial of bitopertin in EPP patients conducted at sites in the United States. Additional analyses of the BEACON and AURORA trials were presented in June 2024 and in December 2024. In both trials, bitopertin significantly reduced the toxic metabolite, protoporphyrin IX, or PPIX, and was associated with improvements in measures of time spent in sunlight and quality of life, demonstrating a clear association between PPIX reduction and clinical endpoints. In addition, bitopertin was generally well-tolerated. All participants in AURORA and BEACON are eligible to participate in HELIOS, an ongoing open-label, long-term extension study of bitopertin in EPP and XLP. We are also planning APOLLO, a randomized, double-blind, placebo-controlled clinical trial of bitopertin in EPP and XLP patients. In our end-of-Phase 2 meeting, the U.S. Food & Drug Administration, or the FDA, agreed with the potential for reduction of PPIX to serve as a surrogate endpoint to support a potential accelerated approval of bitopertin in EPP and XLP. Under the FDA's Accelerated Approval Program, we would have the potential to submit a New Drug Application, or NDA, for bitopertin in EPP and XLP based on our existing data, and we would be required to conduct a post-marketing confirmatory clinical trial. In our Type C meeting with the FDA in December 2024, we aligned with the FDA on the design of our APOLLO post-marketing confirmatory trial. We plan to initiate the APOLLO trial by mid-2025 and anticipate submitting an NDA for accelerated approval of bitopertin in EPP and XLP in the second half of 2025. We have also entered into a collaborative research and development agreement with the National Institutes of Health, or NIH, to conduct a clinical trial of bitopertin in DBA, which began in July 2023. We are planning additional trials of bitopertin in other indications.

DISC-0974 is the lead product candidate in our iron homeostasis portfolio and was licensed from AbbVie Deutschland GmbH & Co. KG, or AbbVie. DISC-0974 is designed to suppress hepcidin production and increase serum iron levels. We completed a Phase 1 clinical trial in healthy volunteers in the U.S. in June 2022 with results showing an acceptable tolerability profile and evidence of target engagement, iron mobilization and augmented erythropoiesis. We initiated a Phase 1b/2 clinical trial in June 2022 in patients with anemia of MF, and initiated a separate Phase 1b/2 clinical trial in February 2023 in patients with non-dialysis dependent CKD and anemia. We presented interim data from both of these trials in December 2023 as well as additional interim data for anemia of MF in June 2024 and non-dialysis dependent CKD and anemia in October 2024, which additional interim data included safety data and changes in hepcidin, iron, and hemoglobin levels for additional patients, as well as longer follow-up. In December 2024, we presented additional analyses of the Phase 1b study in anemia of MF and initiated the open-label Phase 2 portion of this clinical trial in patients with anemia of MF. We expect to report initial data from this Phase 2 trial in the second half of 2025. We also expect to report data from the multiple-ascending dose, or MAD, portion of the Phase 1b trial in patients with non-dialysis dependent CKD and anemia in the second half of 2025. We are also planning additional trials of DISC-0974 in other anemias of inflammation. In addition, we are developing a preclinical anti-hemojuvelin, or HJV, monoclonal antibody, DISC-0998, which also targets hepcidin suppression and was licensed from AbbVie. DISC-0998 is designed to increase serum iron levels and

has an extended serum half-life as compared to DISC-0974. We believe this profile may be desirable in certain subsets of patients with anemia associated with inflammatory diseases.

Lastly, we are developing DISC-3405, a monoclonal antibody against Transmembrane Serine Protease 6, or TMPRSS6, that we licensed from Mabwell Therapeutics, Inc., or Mabwell. DISC-3405 is part of our iron homeostasis portfolio and is designed to induce hepcidin production and reduce serum iron levels. An IND for DISC-3405 was cleared by the FDA, and a Phase 1 clinical trial in healthy adult volunteers was initiated in October 2023. Interim data was presented from the single-ascending dose, or SAD, portion of the Phase 1 clinical trial of DISC-3405 in healthy volunteers in June 2024. We presented data from the MAD portion of the Phase 1 healthy volunteer study of DISC-3405 in December 2024. We expect to develop DISC-3405 for the treatment of PV and other hematologic disorders, and plan to initiate a Phase 2 clinical trial of DISC-3405 in PV in the first half of 2025.

Our Approach

Our goal is to continue to build and advance a portfolio of product candidates that focus on fundamental biological pathways associated with the formation and function of red blood cells. Red blood cells have the essential role of carrying oxygen via hemoglobin to all tissues and organs in the body. The biological processes that are required to maintain normal levels of functional red blood cells are complex, and a variety of congenital and acquired diseases occur due to imbalances or deficiencies in red blood cell formation and function. Two key components needed to support the formation and function of red blood cells are heme and iron. Heme is an essential part of red blood cells, and when complexed into the hemoglobin protein, it performs the vital function of transporting oxygen throughout the body. Iron is a key component of heme, and therefore both iron and heme are required for erythropoiesis, the biological process by which precursor cells in the bone marrow mature to become red blood cells. Based on previously conducted animal models and preclinical and clinical data, we believe our product candidate portfolio, by targeting fundamental pathways in red blood cell biology, has the potential to address a range of hematologic diseases in which modification of iron and heme plays a critical function.

We are focused on therapeutic approaches that modulate heme and iron to address diseases of heme biosynthesis and red blood cell production, and we target therapeutic mechanisms that have been validated in humans, through evidence from either human genetics or third-party clinical trials. For example, our lead program, bitopertin, which has been evaluated in over 4,000 individuals, has demonstrated suppression of heme biosynthesis in multiple clinical trials conducted by Roche. The targets of our iron homeostasis portfolio, HJV and TMPRSS6, have both been genetically validated in humans and shown to have a role in the regulation of hepcidin and iron homeostasis. For example, individuals with inherited loss of the HJV gene exhibit low levels of hepcidin and individuals with inherited loss of the TMPRSS6 gene exhibit elevated levels of hepcidin.

By focusing on fundamental red blood cell biology that is validated in humans, we believe that our product candidates are more likely to exhibit well-defined biological effects in clinical trials and have the potential for broad applicability across a wide range of hematologic diseases. Our current pipeline is focused on the following three approaches:

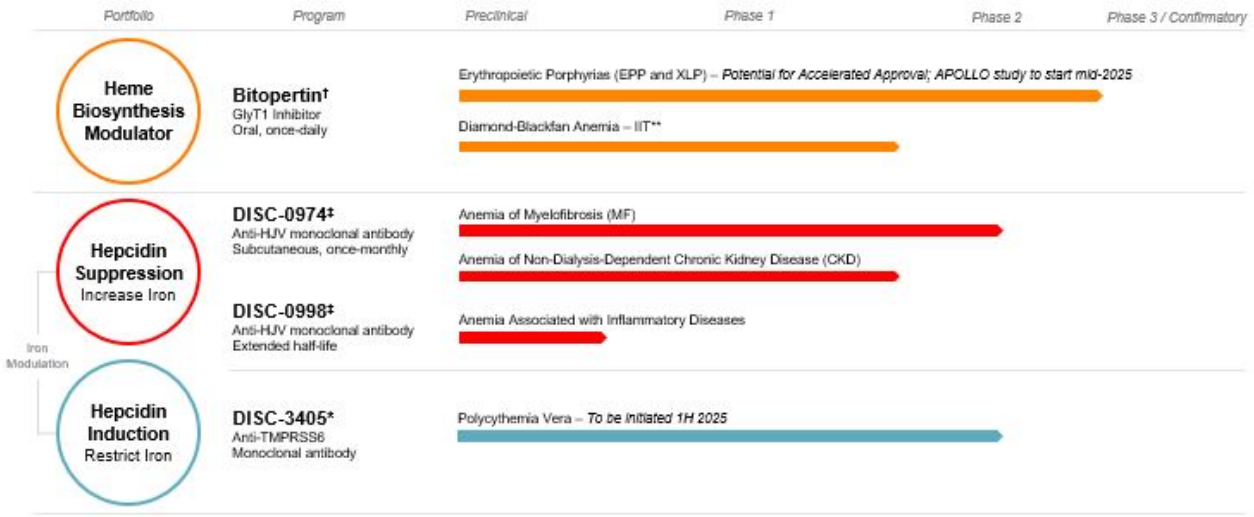
- Modulating the heme biosynthesis pathway, which is anticipated to be useful in diseases caused by excesses in toxic heme pathway metabolites, e.g., EPs;
- Increasing iron availability to red blood cell precursors, which is anticipated to have direct effects on increasing red blood cell production to correct anemia in diseases of iron restriction, e.g., anemia associated with inflammatory diseases; and
- Decreasing iron availability, which is anticipated to lower red blood cell production in diseases of excessive red cell production, e.g., PV.

Our Pipeline

We are building an innovative pipeline of product candidates that aim to modify fundamental biological pathways associated with the formation and function of red blood cells. We have obtained exclusive, worldwide licenses for the development and commercialization of bitopertin, DISC-0974, and DISC-0998, and exclusive rights for DISC-3405 and related antibodies in all territories outside of Greater China and Southeast Asia.

Clinical-Stage Product Candidates

The diagram below reflects the status of the clinical-stage product candidates, bitopertin, DISC-0974 and DISC-3405, and clinical trials that have been completed, are ongoing or are expected to initiate by the end of 2025. The timelines shown reflect our current expectations and beliefs based on our internal plans and regulatory interactions to date.



†Bitopertin in-licensed from Roche; ‡ DISC-0974 and DISC-0998 in-licensed from AbbVie; *DISC-3405 in-licensed from Mabwell (formerly MWTX-003); **Investigator initiated trial with the NIH

We also plan to develop bitopertin, DISC-0974 and DISC-3405 for other indications. For example, we are exploring the potential of bitopertin as a treatment for macrocytic anemias, such as DBA and certain types of myelodysplastic syndromes, or MDS, in preclinical studies and have entered into a collaborative research and development agreement with the NIH to conduct an NIH-sponsored clinical trial of bitopertin in DBA. The trial began in July 2023.

Preclinical-Stage Product Candidates

We also have preclinical-stage programs in development. This includes DISC-0998: a separate, preclinical anti-HJV monoclonal antibody, which is also designed to target hepcidin suppression and was licensed from AbbVie. DISC-0998 is designed to increase serum iron levels and has an extended serum half-life as compared to DISC-0974. We believe this profile may be desirable in certain subsets of patients with anemia associated with inflammatory diseases.

Our Strategy

Our mission is to significantly improve the lives of patients suffering from hematologic diseases by developing differentiated product candidates, including ones designed to target fundamental pathways associated with the formation and function of red blood cells. To achieve our mission, we are focused on the following key elements of our strategy:

- **Obtain regulatory approval for and successfully commercialize bitopertin for the treatment of patients with EPP and XLP, and expand into other diseases characterized by dysregulation of the heme biosynthesis pathway.** In multiple clinical trials conducted by Roche in other indications, bitopertin was observed to be a regulator of heme biosynthesis. We are initially developing bitopertin for the treatment of patients with EPP and XLP, which are part of a group of severe diseases, known as porphyrias, caused by defects in the heme biosynthesis pathway that cause an accumulation of toxic metabolites referred to as porphyrins. Based on the clinical data generated by Roche in multiple clinical trials in other indications, the compelling preclinical data we have generated, and the results of our BEACON and AURORA Phase 2 clinical trials, we believe bitopertin has the potential to be a disease-modifying treatment for these

patients. In our end-of-Phase 2 meeting, the FDA agreed with the potential for reduction of PPIX to serve as a surrogate endpoint to support a potential accelerated approval of bitopertin in EPP and XLP. Under the FDA's Accelerated Approval Program, we would have the potential to submit an NDA for bitopertin in EPP and XLP based on our existing data, and we would be required to conduct a post-marketing confirmatory clinical trial. In our Type C meeting with the FDA in December 2024, we aligned with the FDA on the design of our APOLLO post-marketing confirmatory trial. We plan to initiate the APOLLO trial by mid-2025 and anticipate submitting an NDA for accelerated approval of bitopertin in EPP and XLP in the second half of 2025. We also plan to explore the potential of bitopertin to treat other hematologic diseases, including DBA.

- **Advance the clinical development of DISC-0974 for the treatment of anemia associated with MF, CKD and other inflammatory diseases.** We are initially developing our lead hepcidin-suppressing program, DISC-0974, for the treatment of anemia associated with MF and CKD. Our Phase 1, placebo-controlled, single-ascending dose clinical trial of DISC-0974 in healthy volunteers showed an acceptable tolerability profile and evidence of target engagement and iron mobilization and augmented erythropoiesis. We initiated a Phase 1b/2 clinical trial in June 2022 in patients with anemia of MF and initiated a separate Phase 1b/2 clinical trial in February 2023 in patients with non-dialysis dependent CKD and anemia. We presented interim data from both of these trials in December 2023 as well as additional interim data for anemia of MF in June 2024 and non-dialysis dependent CKD and anemia in October 2024. In December 2024, we presented additional analyses of the Phase 1b study in anemia of MF and initiated the open-label Phase 2 portion of this clinical trial in patients with anemia of MF. We expect to report initial data from this Phase 2 trial in the second half of 2025 and final data in 2026. We also expect to report data from the MAD portion of the Phase 1b trial in patients with non-dialysis dependent CKD and anemia in the second half of 2025. Subject to the results of such MAD portion of the study, we expect to initiate and report initial data from a Phase 2 clinical trial in patients with non-dialysis dependent CKD and anemia in 2026. We also plan to further expand the development of DISC-0974 into anemias associated with other inflammatory diseases, such as inflammatory bowel disease.
- **Advance the clinical development of DISC-3405 for the treatment of PV and expand into other diseases associated with excess iron availability.** The second program in our iron homeostasis portfolio is DISC-3405, which is designed to induce hepcidin production. The inhibition of TMPRSS6 has been shown in non-clinical studies to increase hepcidin levels and restrict iron availability, and DISC-3405 demonstrated activity in disease models of PV and beta-thalassemia. In clinical trials conducted by third parties, iron restriction through a hepcidin mechanism resulted in disease control in patients with PV. We presented interim data from the SAD portion of our Phase 1 clinical trial of DISC-3405 in healthy volunteers in June 2024 and presented data from the MAD portion of such study in December 2024. We expect to develop DISC-3405 for the treatment of PV and other hematologic disorders, and plan to initiate a Phase 2 clinical trial of DISC-3405 in PV in the first half of 2025 and report data in 2026.
- **Continue to build our pipeline through internal research or business development.** Though we have yet to generate complete clinical data for each of our product candidates, we believe that all of our current product candidates, if approved, could have pipeline-in-a-product potential, and for each product candidate, we plan to explore its potential across multiple hematologic diseases. In addition, we plan to leverage our expertise in hematology to further grow our pipeline through both internal discovery and development of new therapeutic candidates and in-licensing of external assets. This approach includes developing both next-generation programs to support our existing heme biosynthesis and iron homeostasis portfolios as well as molecules that target other pathways associated with red blood cells that may be of strategic and biological interest. For example, we are developing DISC-0998, a preclinical monoclonal antibody as a next generation product candidate against HJV, the same target as DISC-0974. We believe DISC-0998 has improved pharmacokinetic, or PK, and pharmacodynamic, or PD, properties that may benefit certain subsets of patients with anemia associated with inflammatory diseases.
- **Opportunistically evaluate strategic collaborations to maximize the value of our product candidates and preclinical programs.** We have obtained exclusive, worldwide licenses for the development and commercialization of bitopertin, DISC-0974, and DISC-0998, and exclusive rights for DISC-3405 (formerly MWTX-003) and related antibodies in all territories outside of Greater China and Southeast Asia. As we advance the development of our product candidates and preclinical programs across multiple indications and continue to generate additional data, we intend to continuously evaluate our options for maximizing the value of our overall portfolio. For example, in certain geographies, we may opportunistically enter into strategic collaborations to accelerate the development and maximize the commercial potential of any or all of our product candidates or preclinical programs. For each product candidate, preclinical program, indication, and geographic region, our goal is to find the best path forward for the development of our product candidates and preclinical programs in order to treat patients in need of new therapies, while also maximizing value for our stockholders.

Our Corporate History and Team

We were founded in 2017 with the mission to design, develop, and commercialize medicines for patients with hematologic diseases. Since inception, we have focused on building our pipeline of product candidates through both internal drug discovery activities and external business development, conducting preclinical studies and clinical trials, preparing for the potential commercialization of bitopertin, if approved, and establishing and maintaining our intellectual property portfolio.

We have assembled a management team with extensive experience in successfully developing, manufacturing, and commercializing transformative therapies as well as in business development and alliance management. Collectively, our team led, or was involved in, the development, regulatory approval, and commercialization of therapies for hematologic diseases, such as Idhifa, Reblozyl, Pyrukynd, Adynovate, Tibsovo, Casgevy, and Roxadustat, as well as numerous late-stage clinical and approved therapies for other therapeutic areas. Our team has significant previous experience at leading biotechnology and pharmaceutical companies, including Acceleron Pharma, Inc., Agios Pharmaceuticals, Inc., Albireo Pharma, Inc., Bristol-Myers Squibb Company, CRISPR Therapeutics AG, FibroGen, Inc., Genzyme Corporation, Johnson & Johnson, Merck & Co., Inc., Pfizer Inc., Replimune Group Inc., Takeda Pharmaceutical Co., Vertex Pharmaceuticals Incorporated, and The Medicines Company. Our management team's wide-ranging expertise in rare diseases, hematology, medicinal chemistry, protein biochemistry, and clinical development provide a singular vision for building a company focused on fundamental mechanisms to develop treatments for patients with hematologic diseases.

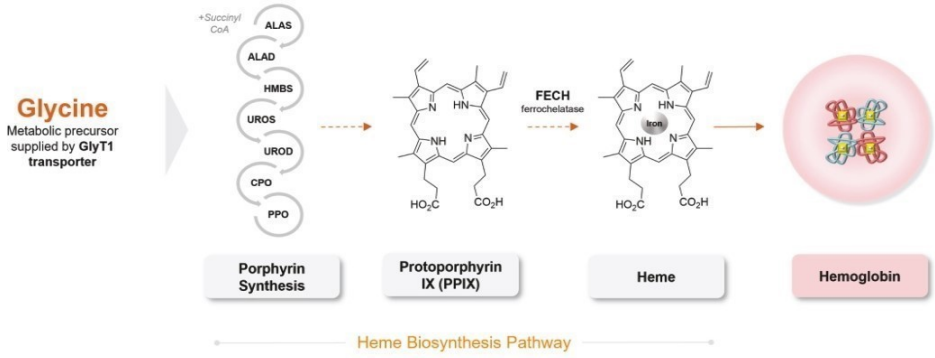
Our Heme Biosynthesis Modulation Portfolio

Our first therapeutic approach is focused on the treatment of diseases caused by defects in heme biosynthesis, a multistep enzymatic process that is critical for the formation of new red blood cells. Heme is an essential part of red blood cells, and when complexed into the hemoglobin protein, it performs the vital function of transporting oxygen throughout the body. However, genetic or acquired defects in the enzymes that mediate heme biosynthesis, as well as deficiencies in the incorporation of heme into hemoglobin, can result in the accumulation of toxic metabolites, leading to a range of hematologic diseases.

Heme Biosynthesis: Fundamental to Erythropoiesis

Erythropoiesis is the biological process by which precursor cells in the bone marrow mature to become red blood cells. The primary function of red blood cells is to transport oxygen throughout the body. Hemoglobin, an iron-containing protein found in all red blood cells, is responsible for binding to oxygen in the lungs, transporting it throughout the body and releasing it in peripheral tissues. The key oxygen binding function of hemoglobin is mediated by its heme component, a molecular complex comprising a porphyrin molecule and iron. Because red blood cells consist largely of heme-containing hemoglobin, newly forming red blood cells must synthesize tremendous amounts of heme. Heme biosynthesis is a complex process that begins with the amino acid glycine and requires multiple subsequent enzymatic reactions, as shown in the figure below. Heme is a highly reactive and potentially toxic complex, as are many of the porphyrin molecules that are generated as metabolic intermediates during heme biosynthesis. As a result, heme biosynthesis is tightly regulated to avoid a build-up of free heme or porphyrins. As new red blood cells are forming in the bone marrow, the heme biosynthesis pathway is tightly coordinated with the expression of the protein subunits of hemoglobin, the globins, and the uptake of iron. The vast majority of newly synthesized heme is incorporated into hemoglobin and does not accumulate in free form to toxic levels. Moreover, the entire process of erythropoiesis is regulated by the availability of heme. As a result, agents that affect heme biosynthesis have broad potential to treat diseases of the heme and hemoglobin biosynthesis pathways and other hematologic diseases resulting from dysregulated erythropoiesis.

Overview of the Heme Biosynthesis Process - Eight Enzymatic Steps from Glycine to Heme



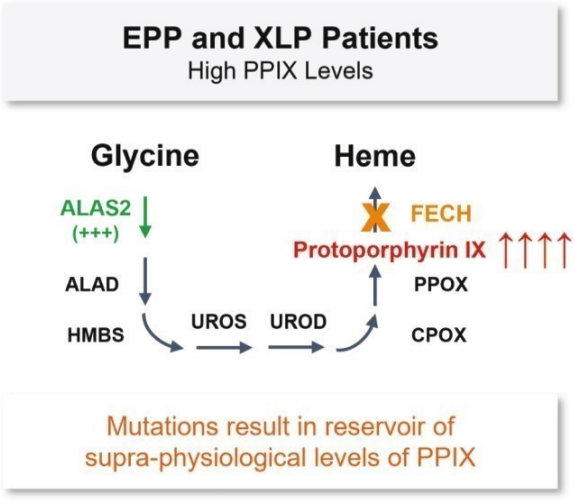
Heme Biosynthesis as a Therapeutic Target for Diseases

In many hematologic diseases, there is abnormal proliferation and differentiation of the progenitor cells that develop into red blood cells. An alteration in any aspect of red blood cell maturation can result in the build-up of metabolic intermediates from heme and hemoglobin biosynthesis, and these intermediates can cause a variety of disease states. Defects of the heme biosynthesis enzymes in the erythroid lineage can cause the build-up of metabolic intermediates called porphyrins and lead to a set of diseases referred to as EPs. In EPs, porphyrins accumulate to inappropriately high levels and cause damage, particularly in the skin, gallbladder, and liver. Similarly, defects in globin biosynthesis, often caused by mutations in the ribosomes that are necessary for mediating globin biosynthesis, result in the build-up of heme that is not complexed with globin. This free heme can damage newly forming red blood cells, leading to forms of anemia observed in DBA and in certain types of MDS. In diseases characterized by defects in the genes coding for the globins, such as sickle cell disease and beta thalassemia, the reduction of heme biosynthesis has the potential to reduce the production of pathologically altered globins that aggregate or polymerize, causing oxidative damage and hemolysis. In people without globin abnormalities, excessive production of red blood cells with normal hemoglobin can cause PV, in which the higher hematocrit can lead to thrombotic disease, including stroke. Restricting heme formation has the potential to ameliorate symptoms in certain patients with these hemoglobinopathies and disorders of red blood cell excess. Therefore, we believe that inhibitors of heme biosynthesis have the potential to treat a wide range of hematologic diseases by restricting the production of damaging metabolites, including porphyrins, heme and globins.

Erythropoietic Porphyrias

EPs are a family of rare, debilitating, and potentially life-threatening diseases caused by mutations that affect the heme biosynthesis pathway. These mutations result in the toxic accumulation of metabolic intermediates in the blood called porphyrins, which can absorb light through the skin and mediate the generation of toxic reactive oxygen species that cause damage to the skin and other tissues. Consequently, when patients with porphyria are exposed to sunlight, they experience excruciating pain, blistering, and edema in the skin. This severe phototoxicity often results in a lifelong aversion to and fear of light, which has a negative impact on patients and their families, particularly for young children. These effects include impaired psychosocial development and conditions, such as anxiety, depression, and social isolation that may require significant adjustments to career and other life choices. EPs comprise three subtypes that are each linked to a specific mutation or deficiency in one of the enzymes in the heme biosynthesis pathway: (1) EPP, which is linked to the ferrochelatase, or FECH, enzyme; (2) XLP, which is linked to the delta-aminolevulinic acid synthase-2, or ALAS2, enzyme; and (3) congenital erythropoietic porphyria, or CEP, which is linked to the uroporphyrinogen III cosynthase, or UROS, enzyme. As shown below, mutations in the FECH and ALAS2 enzymes lead to a pathological accumulation of PPIX, and as a result, patients with EPP or XLP typically have high levels of PPIX.

Genetic and Biochemical Basis for EPP and XLP: FECH and ALAS2 Mutations Increase PPIX Levels



Figures adapted from Halloy et al. (2021) *Cell Chem Biol*

EPP is a rare, inherited metabolic disease characterized by a deficiency of the FECH enzyme. FECH is responsible for the last step in heme biosynthesis and catalyzes the insertion of iron into PPIX to create the final heme moiety. In patients with EPP who have abnormally low levels of FECH, excessive amounts of PPIX accumulate in the bone marrow, blood plasma, and red blood cells. This accumulation of PPIX, which becomes highly reactive and toxic when exposed to light, causes the hallmark EPP symptom of photosensitivity, or skin hypersensitivity to sunlight and some types of artificial light, such as fluorescent lights. After

exposure to light, the patient's skin may initially become itchy and red, and then affected individuals often experience a severe burning sensation that may persist for days. PPIX also accumulates in the gallbladder and liver and causes complications in these organs for some patients. An estimated 25% of patients may develop gallstones that require surgical removal. Many patients live with subclinical liver damage, which progresses to overt liver failure and requires liver transplant in approximately 2% to 5% of patients. The onset of symptoms affecting the skin usually occurs in early childhood; however, in some cases, onset may not occur until adolescence or adulthood. EPP has been reported worldwide, with prevalence between 1 in 75,000 to 1 in 200,000, but a recent genetic study suggests that the genetic prevalence may be higher at approximately 1 in 17,000. Recent analyses we completed of medical claims data using the ICD-10 code for EPP suggests there are approximately 14,000 diagnosed patients in the U.S.

XLP is a genetically distinct inherited metabolic disease with a clinical presentation that is similar to EPP. The causative mutation in XLP occurs in the ALAS2 gene, which codes for the first enzyme in the heme biosynthesis pathway that is found on the X chromosome and inherited with an X-linked pattern. The mutation causes increased ALAS2 function, which results in pathologic accumulation of PPIX. XLP affects both males and females, but males usually develop a severe form of the disease. Females with an ALAS2 mutation may also develop the disease, but severity can range from being asymptomatic to a severe form. Similar to EPP, the major symptom of this disease is skin hypersensitivity to sunlight and some types of artificial light. The exact incidence or prevalence of XLP is unknown, but it is often estimated at one-tenth the incidence of EPP. EPP and XLP, when combined, are the third most common porphyria.

CEP, also known as Günther Disease, is the rarest and most severe form of the EPs and results from the deficient function in UROS, the fourth enzyme in the heme biosynthesis pathway. In CEP, the impaired function of this enzyme leads to the accumulation of excessive amounts of certain porphyrins, particularly in the bone marrow, plasma, red blood cells, urine, teeth, and bones. Similar to EPP and XLP, the major symptom of this disease is skin hypersensitivity to sunlight and some types of artificial light. However, in patients with CEP, the photoactivated porphyrins in the skin cause more profound blistering and scarring. Additionally, the accumulation of porphyrins in the bone impairs bone metabolism and can cause bone loss and deformities. CEP is extremely rare and there have been about 220 affected individuals reported to date.

Current Treatment Options for Erythropoietic Porphyrias

There are currently no disease-modifying therapies available to treat EPs other than bone marrow transplantation, which is associated with high rates of morbidity and mortality. Lifestyle alterations to avoid light exposure are the primary approach to managing phototoxicity in EP patients. Sunscreens, tinted windows, and protective clothing are also commonly used in addition to behavioral modifications. The only class of approved therapies for patients with EP are melanocortin 1 receptor agonists, which are designed to promote melanin production, or tanning, and thereby increase patient tolerance to sunlight. Afamelanotide, an a-melanocyte-stimulating hormone analog delivered by a surgically-administered subcutaneous implant, was approved by the FDA in 2019 for the treatment of adults with EPP. Afamelanotide provides reduction in photosensitivity, but is not designed to reduce PPIX production and is associated with side effects, such as nausea, hyperpigmentation and a darkening of or increase in melanocytic nevi. In a pivotal trial, afamelanotide increased the median number of pain free hours in daytime (10am to 6pm) over 180 days from 40.5 hours in a placebo group to 64.1 hours in the treatment group. Another melanocortin 1 receptor agonist, dersimelagon, which is orally administered, is currently in Phase 3 development by a third party. Overall, there remains a significant unmet need despite the use of melanocortin 1 receptor agonists, as they provide incomplete resolution of photosensitivity and more importantly, are not designed to reduce the production of protoporphyrins or address hepatobiliary complications, such as gallstones and progressive liver disease.

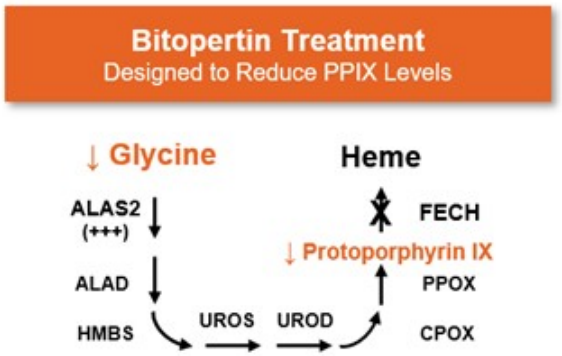
Patients with EP who have progressive liver damage are managed through periodic monitoring, and in cases of liver failure, transplantation is required. While bone marrow transplantation has been used to cure EPs, it is associated with high rates of morbidity and mortality. Therefore, this procedure is usually considered only for younger patients after a liver transplant, for older patients with recurrent disease affecting the liver allograft, or for patients with progressive liver disease.

Our Solution: Bitopertin, an Oral, Selective GlyT1 Inhibitor

Bitopertin is designed to be an oral, selective inhibitor of GlyT1, a key membrane transporter required to supply developing red blood cells with sufficient glycine to support erythropoiesis. By limiting glycine uptake at the first step in heme biosynthesis in newly forming red blood cells, bitopertin is designed to reduce the activity of this pathway, as shown below, and therefore has the potential to treat a range of hematologic disorders associated with the biosynthesis of heme and hemoglobin.

Bitopertin May Treat a Range of Hematologic Disorders

We are initially focused on the ability of bitopertin to suppress the accumulation of PPIX. Based on its mechanism of action, we believe bitopertin has the potential to be a disease-modifying treatment for EPP and XLP.



EPP and XLP are diseases marked by severe photosensitivity and damage to the hepatobiliary system caused by the accumulation of PPIX. PPIX has been well-characterized to absorb light and induce inflammation and tissue damage, manifesting clinically as painful phototoxic reactions. Lower levels of PPIX are associated with lower disease severity. Epidemiologic data correlate increasing PPIX concentrations with decreased light tolerance, and interventions that reduce PPIX in patients correlate directly with increased light tolerance. Lower PPIX levels (by roughly 30-50%) increased light tolerance in patients. 25% of patients with lower PPIX levels experienced symptoms versus 75-100% of patients with medium to high PPIX levels under controlled light exposure. During pregnancy, women with EPP experience temporary disease remissions that increase sunlight tolerance and coincide with a reduction in PPIX levels. For example, in a study conducted by a third-party, pregnant women were observed to have a median reduction of 53% in PPIX levels during pregnancy, resulting in a significant reduction in their EPP symptoms. Disease symptoms return after delivery when PPIX levels revert to pre-pregnancy levels, leading to the hypothesis that the fetus may utilize plasma PPIX as a substrate for its own escalating heme biosynthesis requirements, thus reducing PPIX levels in the mother's bloodstream. In a third-party study of extracorporeal photoinactivation, a process that reduces circulating PPIX levels, symptoms were markedly improved after reduction in blood PPIX concentrations. In this study, blood was removed from the body and illuminated with controlled wavelength light to inactivate PPIX, and the blood in which the PPIX was inactivated was re-infused. This procedure resulted in PPIX reductions of approximately 30% and light tolerance was temporarily increased 14-fold, a level of improvement that is expected to permit near-normal patient lifestyle. However, given the technical complexities associated with this procedure, it has not been widely adopted as a therapeutic option in patients.

We believe that the findings from our preclinical studies and the clinical trials conducted by Roche demonstrate that bitopertin has the potential to act as a durable, and well-tolerated inhibitor of heme biosynthesis in humans. Importantly, we believe these studies support the potential for bitopertin to reduce PPIX to a degree that has, in third-party studies, been associated with marked symptom improvement in patients with EPP and XLP.

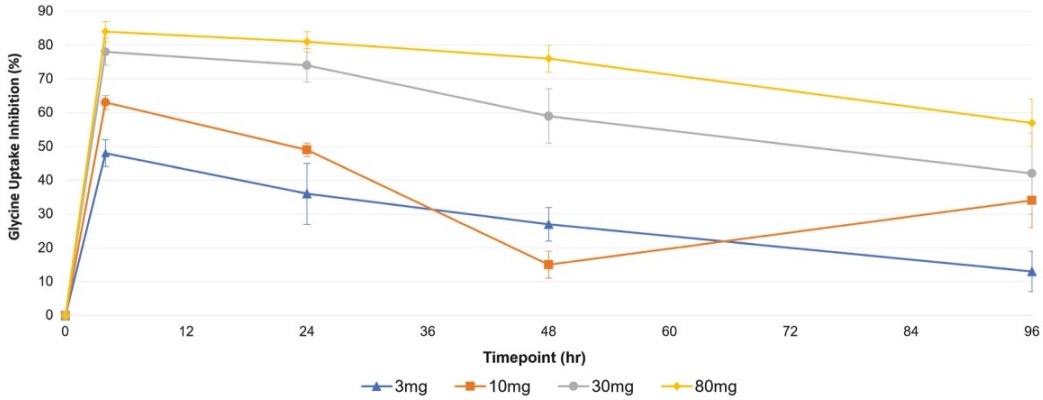
Clinical Data

In May 2021, we licensed exclusive worldwide rights to develop and commercialize bitopertin from Roche. Roche had previously developed bitopertin as a potential therapy for certain symptoms of schizophrenia and obsessive-compulsive disorder, but chose to discontinue the program due to failure to meet primary endpoints in Phase 3 trials for the lead indication after completing over 30 clinical trials in more than 4,000 individuals. Roche conducted a pilot study for the treatment of anemia in 12 patients with beta-thalassemia, a population with a normal heme biosynthesis pathway; this trial did not show consistent increases

in hemoglobin at the doses tested. Despite the observed lack of efficacy, the clinical program established a well-defined and generally well-tolerated profile for bitopertin. Importantly, these trials confirmed that bitopertin inhibits glycine uptake in red blood cells and demonstrated the role of GlyT1 inhibition in heme biosynthesis during red blood cell production. A mild, dose-dependent decrease in heme biosynthesis was observed in multiple clinical trials, which manifested as a decrease in hemoglobin of approximately 0.5 to 2 g/dL that stabilized after approximately 16 weeks, the approximate lifespan of a red blood cell.

For example, a single dose clinical trial in healthy volunteers evaluating bitopertin at doses ranging from 3 mg to 80 mg administered once-daily in 24 individuals demonstrated dose-dependent inhibition of erythrocyte glycine uptake levels, as shown below.

Bitopertin Inhibited Erythrocyte Glycine Uptake in Humans in a Dose-Dependent Manner

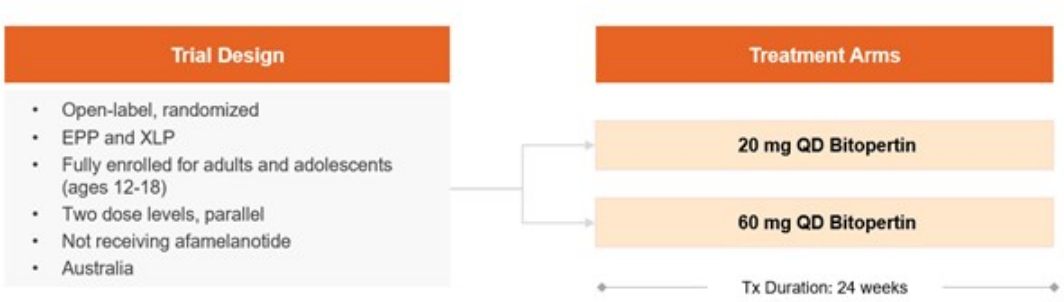


In multiple Phase 3 clinical trials, Roche demonstrated that in patients with schizophrenia who are otherwise hematologically normal, inhibition of glycine uptake resulted in a reduction in hemoglobin production. Patients were administered placebo or bitopertin at 10 mg/day or 20 mg/day dose levels. The effect on hemoglobin was dose-dependent, with patients receiving 10 mg/day and 20 mg/day of bitopertin experiencing a mean decrease in hemoglobin at 52 weeks of approximately 0.5 g/dL and approximately 1.0 g/dL, respectively. The effect of bitopertin on hemoglobin reached a plateau at approximately week 26 and effects were generally stable for the remainder of the 52-week trial.

BEACON: Completed Phase 2 Clinical Trial in Patients with EPP and XLP

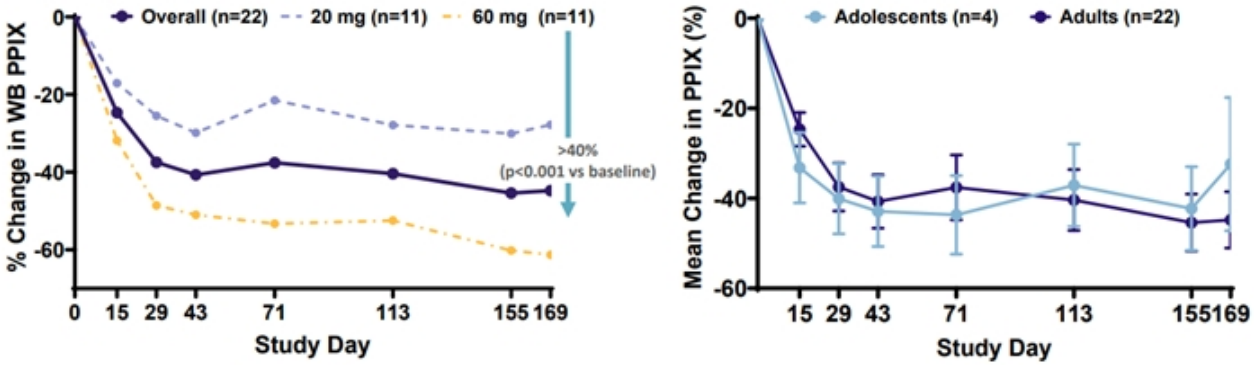
In July 2022, we initiated BEACON, a Phase 2 clinical trial of bitopertin in EPP and XLP patients conducted at sites in Australia. The study was a randomized, open-label, parallel-dose clinical trial designed to evaluate the safety, tolerability, and efficacy of bitopertin. It enrolled 22 adult patients and four adolescent patients with EPP or XLP. The study primarily assessed changes in levels of PPIX as well as the PK profile, safety and tolerability of bitopertin in EPP or XLP patients. It also included measures of photosensitivity, daylight tolerance, pain and exploratory biomarkers of hepatobiliary disease. Patients received orally-administered bitopertin for 24 weeks at doses of either 20 mg once-daily or 60 mg once-daily. These dose levels have a well-understood profile and similar dosage strengths have been shown to provide substantial inhibition of erythroid glycine uptake in the clinical trials conducted by Roche. The trial design is summarized in the figure below. Participants in the BEACON trial are eligible to participate in HELIOS, an ongoing open-label, long-term extension study of bitopertin in EPP and XLP.

BEACON Trial Design: Open-Label, Phase 2 Clinical Trial of Bitopertin in Patients with EPP or XLP (N = 22 adults, N = 4 adolescents)



Interim data from BEACON were presented at the European Hematology Association, or EHA, annual meeting in June 2023 and at the American Society of Hematology, or ASH, annual meeting in December 2023. Additional analyses were presented at the EHA annual meeting in June 2024 and at the ASH annual meeting in December 2024. Twenty-one of the 22 enrolled adults and three of the four enrolled adolescents completed the study. The average age of the adult participants was 44 years, with 64% being female. Bitopertin significantly reduced PPIX in both the adult and adolescent populations, with reductions of whole blood metal-free PPIX of >40% compared to baseline in the overall adult study population, and with greater reductions in the 60 mg group than the 20 mg group. Most of the PPIX reduction occurred in the first 6 weeks of treatment. Overall, there was a 92% reduction in the incidence of patient-reported full phototoxic reactions compared to baseline. The proportion of symptom-free days improved from 33% at baseline to 79% while taking bitopertin. Sunlight tolerance improved in a series of sunlight challenges that participants self-administered weekly, according to the protocol. People with EPP experience prodromes, or early warning symptoms, of an impending full phototoxic reaction. These prodromes are reversible when sunlight exposure is discontinued, and the time until a prodrome starts is an indicator of light tolerance. Combining data from all adult participants showed a >3-fold improvement in average time to prodrome, as compared to baseline ($p<0.001$). The proportion of adult participants who conducted a sunlight challenge that did not elicit a prodrome increased from 7% at baseline to 55% while taking bitopertin. Of the adult participants that completed the treatment period (n=21), nearly all reported improvements in quality of life measures, with 20 of 21 reporting that their EPP was "much better" or "a little better", 19 of 21 reporting that their EPP was "not at all" or "mild" in severity, and 18 of 21 reporting that EPP impacted their quality of life in the last 7 days "not at all" or "a little bit".

BEACON: Primary Endpoint – Percent Change in Whole Blood PPIX



BEACON: Associations between PPIX Reductions and Light Tolerance

Tertiles of PPIX Change

PPIX Increased ← → PPIX Decreased

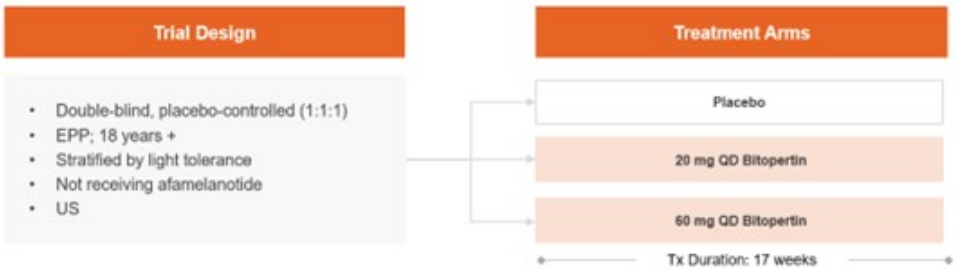
Light Tolerance Measure (Mean ± SD)	Tertile 3 (-43% to 25%)	Tertile 2 (-53% to -43%)	Tertile 1 (-98% to -53%)
Cumulative total time in sunlight without pain (h)	145.6 ± 99.5	190.5 ± 111.6	262.1 ± 160.6
Average time in sunlight without pain (h)	0.86 ± 0.6	1.1 ± 0.7	1.6 ± 1.0
Change from baseline in time to prodrome (min)	85.3 ± 78.8	96.0± 109.0	165.5 ± 128.8

Safety analyses showed no serious adverse events, stable hemoglobin levels, and no anemia adverse events reported. There was one discontinuation due to an adverse event that was assessed as probably related to bitopertin treatment: a Grade 3 headache in an adult participant. Overall, 59% of adult participants and 100% of adolescent participants reported dizziness, 18% of adult participants and zero adolescent participants reported headache, and 14% of adult participants and zero adolescent participants reported nausea.

AURORA: Completed Phase 2 Clinical Trial in Patients with EPP

Separately, in October 2022 we initiated AURORA, a Phase 2, randomized, double-blind, placebo-controlled, parallel dosing trial in 75 adult patients with EPP conducted at sites in the United States. We enrolled patients into a placebo group (n=24), a 20 mg/day dose group (n=26) and a 60 mg/day dose group (n=25), with bitopertin delivered as tablets taken orally once per day for a period of 17 weeks. These dose levels have a well-understood profile and similar dosage strengths have been shown to provide substantial inhibition of erythroid glycine uptake based on the clinical trials conducted by Roche. This trial included assessments of blood PPIX levels, with a primary endpoint of percent change in whole blood metal-free PPIX, and patient photosensitivity, including a secondary endpoint of cumulative total time in sunlight between 10:00 a.m. and 6:00 p.m. on days without pain observed over the 4-month treatment period. The FDA has previously approved afamelanotide for the treatment of photosensitivity in adult EPP patients on the basis of a clinical endpoint measuring a change in pain-free time spent in sunlight in treated patients, relative to patients treated with placebo. Additional study measures included time to prodrome, hepatobiliary markers, quality of life, safety and tolerability, among others. The trial design is summarized in the figure below. Participants in the AURORA trial are eligible to participate in HELIOS, an ongoing open-label, long-term extension study of bitopertin in EPP and XLP.

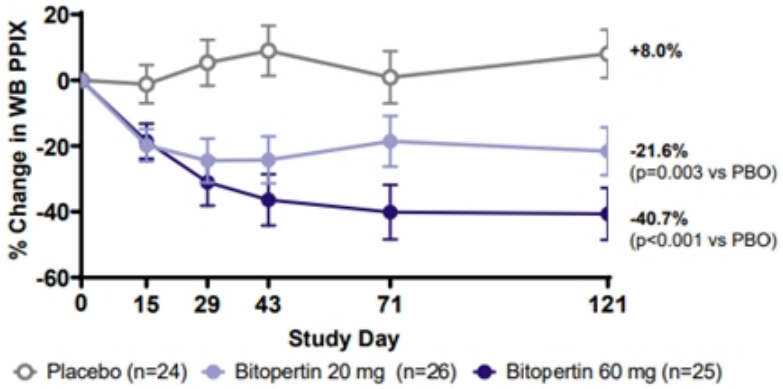
AURORA Trial Design: Randomized, Double-Blind, Placebo-Controlled Phase 2 Clinical Trial of Bitopertin in Patients with EPP (N = 75)



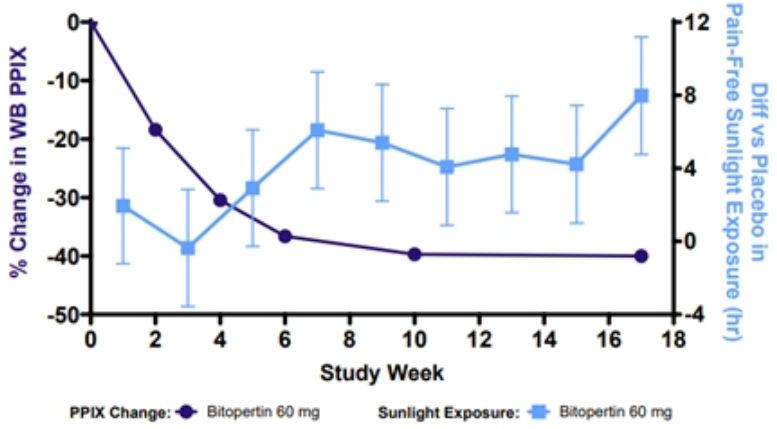
We presented topline data from AURORA in April 2024. Additional analyses were presented at the EHA annual meeting in June 2024 and at the ASH annual meeting in December 2024. Seventy-two of the 75 participants completed the study. With respect to the primary endpoint, bitopertin resulted in significant, dose-dependent, and sustained reductions in whole blood PPIX levels compared to baseline (-21.6% for 20 mg ($p=0.003$ vs placebo) and -40.7% for 60 mg ($p<0.001$ vs placebo); the placebo group had mean increases of +8.0%). As in BEACON, most of the PPIX reduction occurred in the first 6 weeks of treatment. With respect to clinical efficacy endpoints, there were substantial and dose-dependent reductions in phototoxic reactions with pain during the 4-month study period, consisting of a 75% reduction in the incidence rate of new phototoxic reactions with pain at the 60 mg dose

group compared to placebo (p=0.011), and a 60% reduction in the 20 mg dose group compared to placebo (p=0.109). Fewer bitopertin-treated patients reported a phototoxic event compared to placebo (19% for 20 mg and 12% for 60 mg compared to 46% for placebo). There were also dose-dependent improvements in quality-of-life scores as measured by the Patient Global Impression of Change, or PGIC, scale, which were statistically significant for the 60 mg dose: 77% of completers at 20 mg and 86% at 60 mg (p=0.022 vs placebo) reported that their EPP was "much better" compared with 50% for placebo. With respect to cumulative total time in sunlight on days without pain, bitopertin-treated patients recorded a mean of 175.1 hours at 20 mg and 153.1 hours at 60 mg, compared with 133.9 hours for placebo. While the magnitude of the improvement in the bitopertin-treated patients from baseline was comparable to that observed in the BEACON study, the benefit in the placebo arm in the AURORA trial was greater than expected and the results were not statistically significant compared to placebo. In addition, there were large improvements in light tolerance from baseline in 20 mg and 60 mg bitopertin treatment groups as measured by time to prodrome, but the results were not statistically significant relative to placebo. However, a post-hoc longitudinal analysis showed time-dependent improvement in light tolerance that was nominally significant compared to placebo in both the 20 mg (p=0.026) and 60 mg (p=0.013) dose groups, and a two-fold improvement in light tolerance relative to baseline in both 20 mg and 60 mg dose groups. Greater PPIX reductions were associated with improvements in multiple light tolerance measures, including cumulative total time in light, average time in sunlight without pain, change from baseline in time to prodrome, as well as PGIC, and evaluation of the time course of phototoxic reactions and sunlight exposure showed greater treatment effect in the time period after the PPIX nadir was reached, including elimination of observed phototoxic reactions in the 60 mg dose group.

AURORA: Primary Endpoint – Percent Change in Whole Blood PPIX



AURORA: Association between PPIX Change and Clinical Measures with Bitopertin



	Tertiles of PPIX Change PPIX Increased → PPIX Decreased		
Clinical Measure	Tertile 3 (-7% to 191%) n=25 *Placebo, n=17* *Bitopertin 20 mg, n=7* *Bitopertin 60 mg, n=1*	Tertile 2 (-38% to -7%) n=24 *Placebo, n=5* *Bitopertin 20 mg, n=13* *Bitopertin 60 mg, n=6*	Tertile 1 (-89% to -38%) n=24 *Placebo, n=2* *Bitopertin 20 mg, n=6* *Bitopertin 60 mg, n=16*
Cumulative total time in light without pain (mean ± SE, hr)	117.5 ± 16.6	124.5 ± 13.9	**161.1 ± 19.1**
Average daily time in light without pain (mean ± SE, hr)	1.16 ± 0.17	1.20 ± 0.15	**1.61 ± 0.27**
Change from baseline in time to prodrome (mean ± SE, min)	64.1 ± 8.4	109.4 ± 28.5	**117.4 ± 33.2**
Occurrence of phototoxic reaction (n,%)	8 (32%)	9 (38%)	**2 (8%)**
Occurrence of phototoxic reaction in last 60 days (n,%)	4 (16%)	5 (21%)	**1 (4%)**
PGIC Response of 'Much Better' (n,%)	12 (48%)	18 (75%)	**21 (91%)**

Only 57 patients completed sun exposure challenges at baseline and during the study period to calculate change from baseline in time to prodrome (n=20 in tertile 1, n=18 in tertile 2, n=19 in tertile 3).

Bitopertin was generally well tolerated in both dose groups with no serious adverse events and stable hemoglobin levels. Two patients discontinued treatment due to treatment-emergent adverse events, both in the 60 mg dose group and both of which were assessed as possibly related to bitopertin: one due to dizziness and one due to a skin rash. The most common adverse event reported with bitopertin treatment was dizziness: n=4 in the 20 mg dose group and n=11 in the 60 mg dose group, compared with n=4 in the placebo group (median durations of 4.5, 5, and 2 days, respectively).

Additional Safety Data from Selected Clinical Trials Conducted by Roche

The comprehensive data package from Roche's healthy volunteer trials provides further support for bitopertin's tolerability profile. The identified risks established by Roche across the development program are (percentage bitopertin treated vs. percentage placebo treated): headache (9.8% vs. 6.7%), somnolence (5.2% vs. 3.7%), and dizziness (4.2% vs. 3.6%). The results of single dose bitopertin clinical trials in healthy volunteers at doses ranging from 3 mg to 240 mg (n=290) and multiple dose trials at doses ranging from 10 mg to 180 mg daily for 10 to 120 days (n> 360) demonstrated a comprehensive tolerability profile. In one multiple ascending dose trial, reversible blurred vision was observed in 5 subjects (20%) at or above the 80 mg/day dose level. In a four-month pharmacodynamics study, 11.8% of subjects receiving an active dose noted dysphoria/low mood (mostly at 30 mg/day), as compared to 6.3% of placebo, and dermatological adverse events on hands and feet were observed in 15.7% of subjects (mostly at 60 mg/day). In Phase 3 studies, no association with bitopertin was found for dermatological adverse events or adverse events of blurred vision or low mood. The amount of hemoglobin per red blood cell or per reticulocyte decreased in a dose-dependent manner. No hematologic parameter reached a level at which we would expect clinical signs or symptoms. Roche's Phase 3 program in schizophrenia consisted of six Phase 3 clinical trials (total n=2,438) of 5 mg, 10 mg, and 20 mg doses of bitopertin for up to 52 weeks, followed by extension phases. In these trials, bitopertin treatment was not associated with any significant tolerability issues. Most of the adverse events were considered mild or moderate in severity in all trials.

Our NDA Plans for Bitopertin in EPP and XLP and our Planned Post-Marketing Confirmatory Trial (APOLLO)

In our end-of-Phase 2 meeting with the FDA in September 2024, the FDA agreed with the potential for reduction of PPIX to serve as a surrogate endpoint to support a potential accelerated approval of bitopertin in EPP and XLP. Under the FDA's Accelerated Approval Program, we would have the potential to submit an NDA for bitopertin in EPP and XLP based on our existing data, and we would be required to conduct a post-marketing confirmatory clinical trial. In our Type C meeting with the FDA in December 2024, we aligned with the FDA on the design of our APOLLO post-marketing confirmatory trial. Key features of the trial include:

- Co-primary endpoints of average monthly total time in sunlight without pain between 10:00 a.m. and 6:00 p.m. during the last month of the six-month treatment period and percent change from baseline in whole blood metal-free PPIX after six months of treatment;
- Other measures of efficacy such as occurrence of phototoxic reactions, cumulative total pain-free time in sunlight, PGIC, and time to prodrome;
- Selection of 60 mg dose of bitopertin and six-month treatment duration;
- Inclusion of patients aged 12+ with EPP, including XLP; and
- Double-blind, placebo-controlled study with ~150 patients randomized 1:1.

We plan to initiate the APOLLO trial by mid-2025 and plan to include sites in the United States, Canada, Europe and Australia. We anticipate submitting an NDA for accelerated approval of bitopertin in EPP and XLP in the second half of 2025. Based on the anticipated timing of our NDA submission, we expect enrollment for the APOLLO trial to be well underway by the Prescription Drug User Fee Act date for accelerated approval, if granted.

Bitopertin in Additional Indications: Diamond-Blackfan Anemia and Macrocytic Anemias

We believe that bitopertin may be therapeutically beneficial for the treatment of DBA and other anemias that are characterized as macrocytic anemias. DBA is a genetic condition marked by defective erythropoiesis that is usually caused by genetic mutations in genes coding for ribosomal proteins. Clinically, DBA is a lifelong anemia that presents in infancy and has a 25% mortality rate by age 50. Standard therapy includes chronic steroid treatment and/or regular blood transfusions, and hematopoietic stem cell transplantation is the only known cure for DBA. The ribosomal defects in patients with DBA are thought to cause a build-up of free heme in newly forming red blood cells, and this free heme exerts a toxic effect, resulting in poor red blood cell formation and anemia. Inhibitors of heme biosynthesis have shown marked effects in improving red blood cell production in third-party cellular and animal models of DBA. Accordingly, we anticipate that bitopertin may be able to provide relief from anemia and transfusion in patients with DBA by restricting the accumulation of toxic, free heme. Other anemias characterized by ribosomal defects exhibit a similar phenotype and are collectively referred to as macrocytic anemias. An example is the form of MDS characterized by a deletion in the 5q chromosomal locus, or Del(5q) MDS. Heme biosynthesis inhibitors have shown benefits on red blood cell formation in patient-derived cells from patients with Del(5q) MDS, and therefore we expect bitopertin may be therapeutically beneficial in these related conditions. We are continuing to explore the potential of bitopertin in these additional indications in preclinical studies. We have entered into a collaborative research and development agreement with the NIH to conduct an NIH-sponsored clinical trial of bitopertin in DBA. The FDA authorized the clinical trial to proceed and the trial began in July 2023.

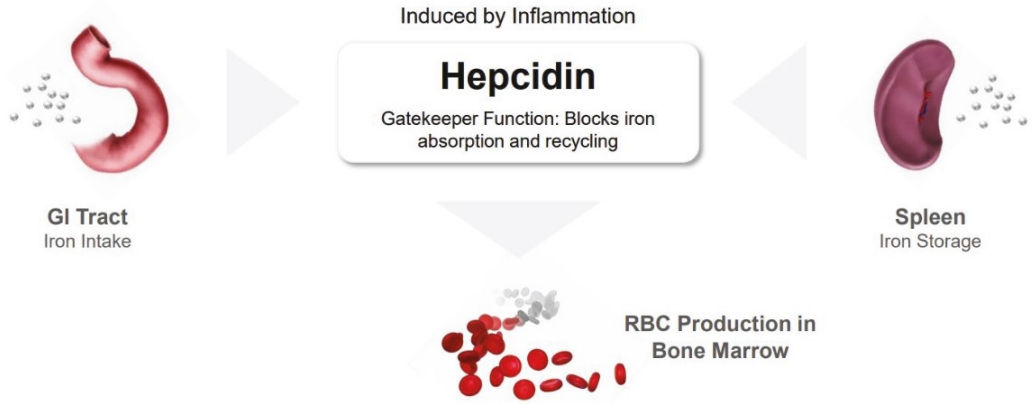
Our Iron Homeostasis Portfolio

In addition to our heme biosynthesis therapeutic approach, we are developing a portfolio of product candidates focused on the modulation of the hepcidin pathway to normalize iron homeostasis. Iron is an essential element that is required for erythropoiesis as well as other important biological functions. Nearly 70% of iron in the human body resides in red blood cells, where it is a fundamental component of hemoglobin, the protein that enables red blood cells to carry and transport oxygen. Although iron is critical to an array of biological functions, excessive levels can be toxic. Consequently, the management of iron levels in the body is a critical and carefully controlled process. Hepcidin is a potent hormone produced in the liver that serves as the primary regulator of iron homeostasis and plays a central role in controlling how iron is absorbed, utilized, stored, and recycled systemically. If this process becomes dysregulated, a wide range of serious, debilitating, and potentially fatal conditions can arise.

Hepcidin: The Master Regulator of Iron Homeostasis

Iron typically enters the body when it is absorbed in the intestine from dietary intake. As it enters circulation, iron is bound to carrier proteins. Iron is a highly reactive metal that can cause oxidative stress and tissue damage in an unbound state. Iron is utilized in target tissues, such as the bone marrow, to support erythropoiesis, and the remaining surplus is directed to specific storage tissues, such as the spleen, where it can be sequestered in specialized macrophages and redeployed when needed. This process is governed by hepcidin, which serves as a gatekeeper in tissues that are a source of iron, both blocking absorption of dietary iron from the intestine and preventing the release of stored iron from the spleen, as shown in the figure below. The body exerts control and responds to demands for iron by increasing or reducing the production of hepcidin, which leads to a reduction or increase in iron availability, respectively.

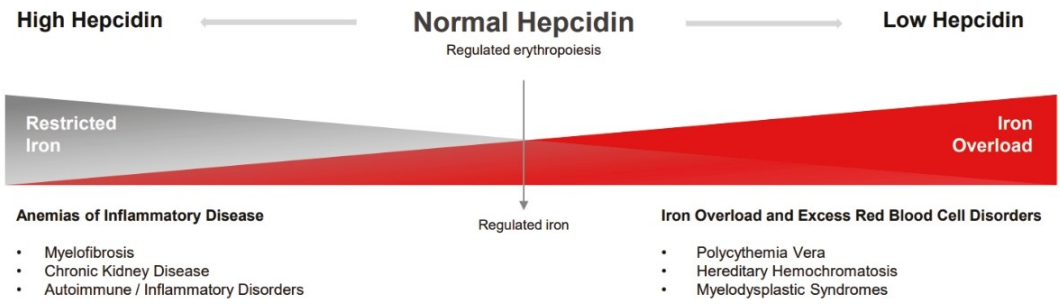
Hepcidin Plays a Central Role in Iron Metabolism and Homeostasis



Hepcidin is a Therapeutic Target for Diseases of Iron Metabolism

Because iron is critical to so many biological functions, particularly in red blood cells, disruptions in its homeostasis, often due to the dysregulated production of hepcidin, can result in a wide range of hematologic diseases, as shown in the figure below. These include diseases that can cause abnormally high production of hepcidin, which deprives developing red blood cells of iron and causes anemia, a frequent complication of cancer, autoimmune conditions, and other inflammatory diseases. Conversely, in certain diseases with abnormally low production of hepcidin, increasing hepcidin and restricting iron availability are expected to provide a therapeutic benefit. For example, in PV, iron restriction through a hepcidin mechanism has been demonstrated to control pathologic production of red blood cells. In other diseases, such as hereditary hemochromatosis, or HH, beta-thalassemia, and MDS, iron levels are pathologically high due to inadequate hepcidin production, and agents that increase hepcidin could be beneficial.

Dysregulated Hepcidin Drives a Wide Range of Hematologic Diseases



We believe that modulating the production of hepcidin to correct pathologic alterations in iron metabolism has the potential to be a powerful therapeutic strategy to address a wide range of diseases. We are leveraging two approaches that are designed to suppress or induce hepcidin production in order to increase or decrease serum iron levels, respectively. Our product candidates target novel pathways whose biological functions have been validated by human genetics and are specific to iron modulation.

Hepcidin Suppression

We are developing a portfolio of product candidates designed to lower hepcidin and restore serum iron levels to address anemia of inflammatory diseases. Our lead product candidate, DISC-0974, is a monoclonal antibody, which we in-licensed from AbbVie, that is designed to inhibit HJV, a critical target for hepcidin production. We selected this target because the effects of inhibiting HJV, namely decreased hepcidin and increased iron availability, have been genetically demonstrated in both animal knockout studies and in patients with juvenile hemochromatosis who lack fully functional genes encoding HJV.

Hepcidin Induction

We are also developing a portfolio of product candidates designed to increase hepcidin and decrease serum iron levels, an approach that has the potential to address a range of diseases where restricting iron would be beneficial, such as excessive red blood cell production in PV and diseases of iron overload. Our lead program, DISC-3405, which we licensed from Mabwell, is a monoclonal antibody designed to inhibit TMPRSS6, a serine protease that normally serves to limit hepcidin production. By inhibiting TMPRSS6, our compounds have the potential to increase production of hepcidin and, in turn, restrict iron availability. We selected this target based on the genetic confirmation of the effects of inhibiting TMPRSS6 in both animal knockout studies and in patients with iron-refractory iron deficiency anemia who lack fully functional genes encoding TMPRSS6.

Our Lead Hepcidin Suppression Program: DISC-0974 For the Treatment of Anemia of Inflammatory Diseases

We are developing DISC-0974, our lead antibody product candidate targeting hepcidin suppression, for the treatment of anemia resulting from iron restriction that typically occurs in the setting of inflammatory diseases. DISC-0974 is designed to be a selective inhibitor of HJV, a bone morphogenetic protein, or BMP, co-receptor. Inflammatory signals, potentiated by BMP signaling, are an underlying cause of elevated levels of hepcidin, leading to low iron bioavailability and subsequent anemia in a broad range of diseases. We believe that abnormally high levels of hepcidin are an important driver of anemia associated with inflammatory diseases and that suppression of hepcidin with DISC-0974 has the potential to provide meaningful benefit in these patients.

Overview of Anemia Associated with Inflammatory Diseases

Anemia of inflammation is a hallmark of a wide range of autoimmune and chronic diseases, including MF, CKD, rheumatoid arthritis, inflammatory bowel disease, cancer, obesity, chronic obstructive pulmonary disease, and cardiovascular disease. Anemia occurs frequently in these diseases and for example, affects approximately 87% of myelofibrosis, 17-50% of chronic kidney disease, 25-35% of inflammatory bowel disease, 35-80% of cancer, and 50% of lupus patients. It is a common cause of chronic anemia and has been estimated to affect over one billion individuals worldwide. This type of anemia is caused by the sustained inflammation associated with these diseases, which produces a host of pro-inflammatory cytokines that impair erythropoiesis. Importantly, these cytokines have an impact on iron homeostasis by inducing the production of hepcidin, which in turn deprives developing erythrocytes of iron. There are currently no approved therapies designed to primarily lower hepcidin, and most patients remain anemic or untreated.

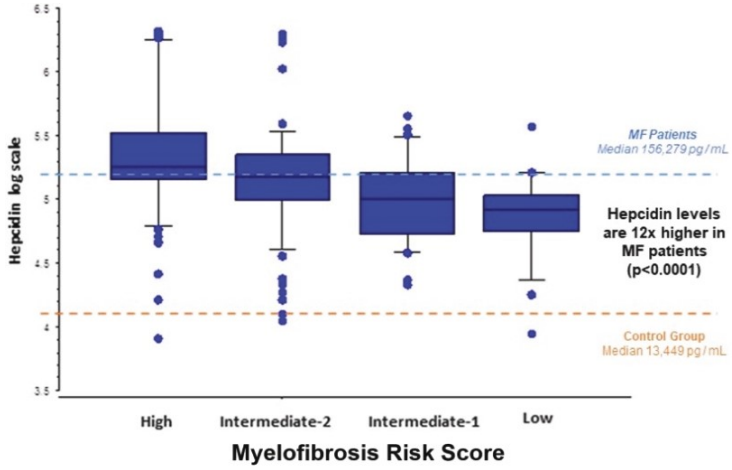
Anemia of Myelofibrosis

MF is a rare, chronic blood cancer that currently affects an estimated 25,000 patients in the United States. It is characterized by progressive fibrosis of the bone marrow brought on by the proliferation of cytokine-producing myeloid cells, which creates a state of chronic inflammation. Severe, progressive, and treatment- resistant anemia is the primary clinical manifestation of MF, and a study in over 200 patients at the Mayo Clinic showed that hepcidin is elevated by approximately 12-fold in these patients, as shown below. Elevated hepcidin levels are correlated with disease severity, anemia, and the need for red blood cell transfusions.

At diagnosis, approximately 87% of patients with MF have anemia, which progressively worsens over time and ultimately renders the majority of patients dependent on chronic red blood cell transfusions. In a study conducted by the Mayo Clinic, within a year of diagnosis, 58% of patients with MF had severe anemia, defined as hemoglobin levels of less than 10 g/dL, and 46% were transfusion-dependent, meaning they required regular transfusion therapy, as shown below. Moreover, existing treatments, such as erythropoiesis-stimulating agents, or ESAs, androgens, corticosteroids, immunomodulators, and splenectomy, are generally viewed as providing minimally effective or inconsistent results, are associated with safety concerns, and do not directly target hepcidin. This is in contrast to the effects observed in a recently published study of a hepcidin-targeted agent conducted in patients with advanced, transfusion-dependent myelofibrosis. In this clinical trial, a partial reduction of hepcidin levels led to approximately 85% of patients having lower transfusion requirements, 41% of patients becoming transfusion independent, increased hemoglobin and improved markers of iron homeostasis.

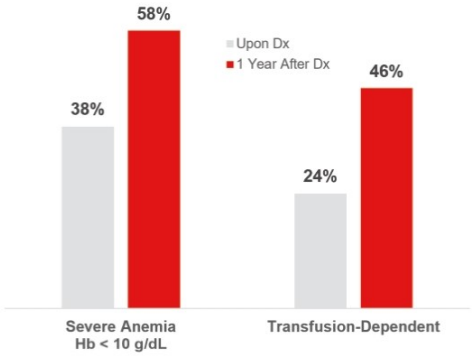
Currently, patients with MF are treated with Janus Kinase, or JAK, inhibitors approved to treat intermediate or high risk MF, including ruxolitinib and fedratinib, which reduce splenomegaly and other symptoms, but typically worsen anemia to the point that patients frequently discontinue treatment.

Elevated Hepcidin Levels in Patients with MF



Adapted from Pardanani et al. (2013) *Am. J. Hematol*

Anemia of MF is Progressive and Severe



Data from Teferri et al. (2012) *Mayo Clinic Proc*

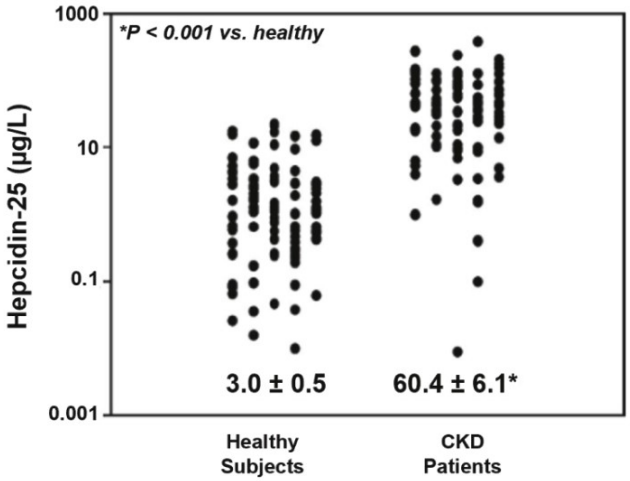
Anemia of Chronic Kidney Disease

CKD is a highly prevalent disease characterized by the progressive loss of kidney function that eventually leads to kidney failure or end-stage renal disease necessitating dialysis or a kidney transplant for survival. It is caused by a constellation of underlying chronic conditions, such as diabetes, hypertension, and heart disease, that damage the kidneys over time and create a chronically inflamed state. CKD is widespread and is estimated to affect nearly 700 million patients worldwide. While it is most common in developed countries, CKD cases are growing rapidly in populous, emerging markets, such as China and India. In the U.S. alone, there are an estimated 39 million patients with CKD, the vast majority of which have not initiated dialysis.

Anemia is a hallmark of CKD and both worsens and becomes increasingly common as kidney function deteriorates. It is associated with increased risk of hospitalization, cardiovascular complications, and death, and frequently causes significant fatigue, cognitive dysfunction, and declining quality of life. The prevalence of anemia in CKD varies depending on the stage of disease and ranges from approximately 17% to 50% in patients with earlier-stage CKD who do not require dialysis to nearly all patients with end-stage renal disease who are dialysis-dependent.

While the underlying cause of anemia of CKD is multifactorial, among the primary molecular drivers are declining production by kidney cells of erythropoietin, or EPO, a growth factor that normally stimulates red blood cell production, and elevated hepcidin levels, which suppress the iron supply needed to support erythropoiesis. Hepcidin levels are correlated with CKD disease stage and severity of anemia and can be nearly 20-fold higher in patients with CKD than in healthy individuals, as shown in the graph below. Hepcidin elevation results from dysregulated overproduction induced by chronic inflammation and accumulation as the body is unable to excrete hepcidin from the kidney. This combination results in a cycle where patients become progressively more anemic and incapable of erythropoiesis as their disease progresses.

Hepcidin Levels Are Elevated in Patients with CKD



Historically, the treatment of anemia of CKD has relied on red blood cell transfusions, but risks associated with iron overload, infection, and the development of antibodies precluding the ability to receive organ transplants have reduced the use of transfusions over time. Beginning in the 1990s, the standard of care shifted to injectable recombinant ESAs, such as EPOGEN (epoetin alfa) and Aranesp (darbepoetin alfa), which are administered to provide supraphysiological levels of erythropoietin to stimulate production of red blood cells. While hemoglobin levels were raised, several large clinical studies conducted by others revealed significant safety risks with the ESAs, including thrombosis, stroke, myocardial infarction, and death, which led to regulatory actions, including a black box warning and other label restrictions. In addition, changes in reimbursement and clinical practice guidelines have all significantly curtailed the use of ESAs for the treatment of anemia of CKD. As a result, a high proportion of patients with anemia of CKD today are either untreated or sub-optimally treated, despite being severely anemic. For example, according to the U.S. Renal Data System, the mean hemoglobin levels of patients who are about to initiate dialysis treatment is 9.3 g/dL, which is significantly below the normal range.

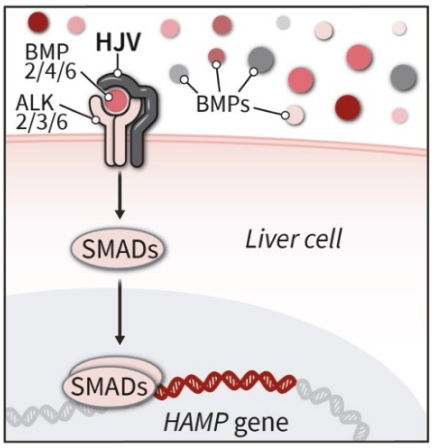
Our Solution: DISC-0974, an Anti-HJV Monoclonal Antibody

DISC-0974 is designed to be an injectable, selective monoclonal antibody targeting HJV, a co-receptor required for hepcidin expression. In multiple preclinical studies, we have demonstrated that DISC-0974 suppressed endogenous production of hepcidin and, as a consequence, increased serum iron levels. Based on this early confirmation of its mechanism, we believe DISC-0974 has the potential to treat a wide range of anemias associated with inflammatory diseases where hepcidin levels are pathologically elevated and serum iron levels for erythropoiesis are restricted. Based on data from our IND-enabling studies, we intend to develop DISC-0974 as a once-monthly, subcutaneous injection.

Hemojuvelin Has a Critical and Specific Role for Hepcidin Regulation and Homeostasis

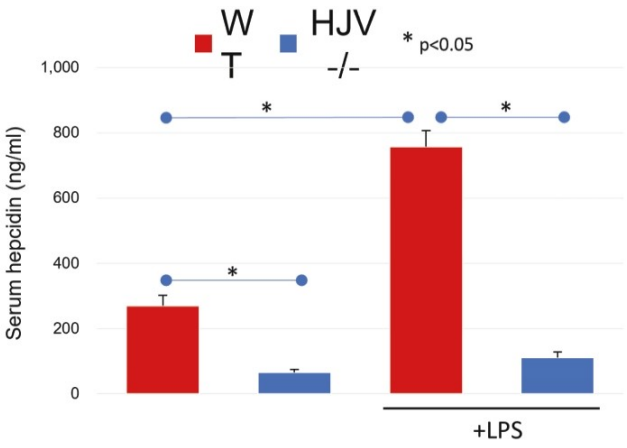
HJV, also called repulsive guidance molecule-c, is a cell surface co-receptor that is primarily expressed in the liver and other tissues with a significant role in iron metabolism, such as skeletal muscle, and is critical for hepcidin production. Signaling through the HJV pathway involves a complex of ligands of the TGF-b superfamily (BMP2/4/6) and other receptors (ALK2/3/6) that induce SMAD phosphorylation and hepcidin (HAMP gene) expression, as shown below. Many components of the BMP signaling pathway are expressed in tissues throughout the body and participate in a range of biological processes, including bone formation and immune cell production. As a result, therapeutic efforts to control hepcidin by targeting the ALK receptors or BMP ligands may affect other tissues and result in off-target side effects. However, based on the phenotype caused by the genetic loss of function of HJV in rodents and humans, we believe that the role of HJV is restricted to iron homeostasis and hepcidin expression, and therefore, we believe that targeting HJV has the potential to result in an improved risk-benefit profile as compared to targeting other members of the BMP pathway.

Hemojuvelin is a Critical and Specific Target for Hepcidin Expression



The importance of HJV in hepcidin expression and iron homeostasis was established through genetic studies in both animals and humans. Specifically, mutations that result in a partial or complete lack of HJV result in significantly reduced hepcidin production and are phenotypically indistinguishable from loss-of-function mutations in hepcidin itself. For example, in a study in mice conducted by a third-party, a knockout of the HJV gene resulted in significantly reduced hepcidin levels in untreated animals as well as in animals challenged with LPS, an inflammatory stimulus, as compared to mice with a functional HJV gene, as shown below.

HJV Gene Knockout in Mice Resulted in Significantly Reduced Hepcidin Levels



Adapted from Fillebeen et al. (2018) *Blood*

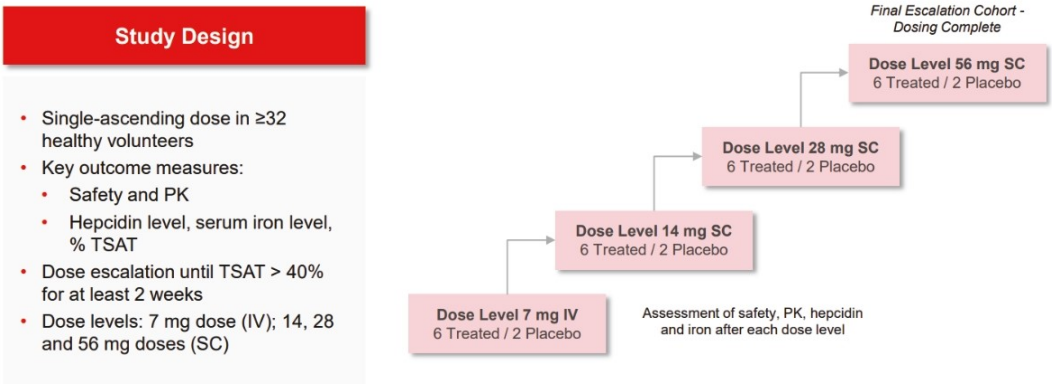
In addition, mutations in the HJV gene in humans markedly reduce hepcidin expression in the liver and result in juvenile hemochromatosis, the most severe form of diseases of iron overload. This genetic evidence suggests that the function of HJV is specific to hepcidin and iron regulation. We believe this specificity is an important attribute in selecting HJV as a target and may result in an improved therapeutic outcome by avoiding unwanted side effects that can result from systemic changes in TGF-b superfamily signaling, such as changes in bone mineral density and immune function. By targeting HJV to reduce hepcidin production, we believe that DISC-0974 has the potential to normalize serum iron levels and restore the production of red blood cells, thereby addressing a key underlying driver of anemia of inflammatory diseases.

Completed Phase 1 Clinical Trial

In July 2021, we initiated a first-in-human, Phase 1, single ascending dose, randomized, double-blind, placebo-controlled clinical trial of DISC-0974 in healthy volunteers to evaluate safety, tolerability, PK, and PD markers such as hepcidin, serum iron levels, TSAT and measures of erythropoiesis. In the initial cohort of the Phase 1 trial, DISC-0974 was administered intravenously.

Subsequent cohorts were dosed with DISC-0974 by subcutaneous administration, which has been shown to be comparable and well-tolerated as compared to intravenous administration in preclinical studies. The trial design is summarized in the figure below.

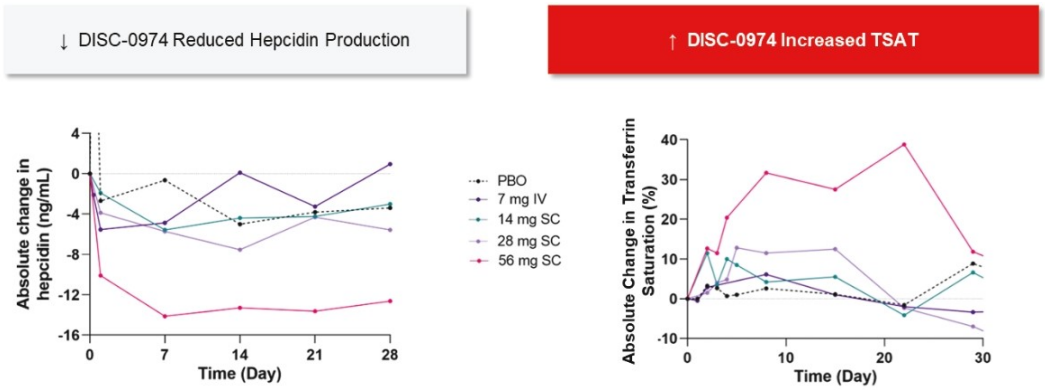
DISC-0974 Phase 1 Clinical Trial Design



We have completed this Phase 1 clinical trial. Data from the Phase 1 clinical trial showed an acceptable tolerability profile and evidence of target engagement and iron mobilization and augmented erythropoiesis. Additional data are discussed below.

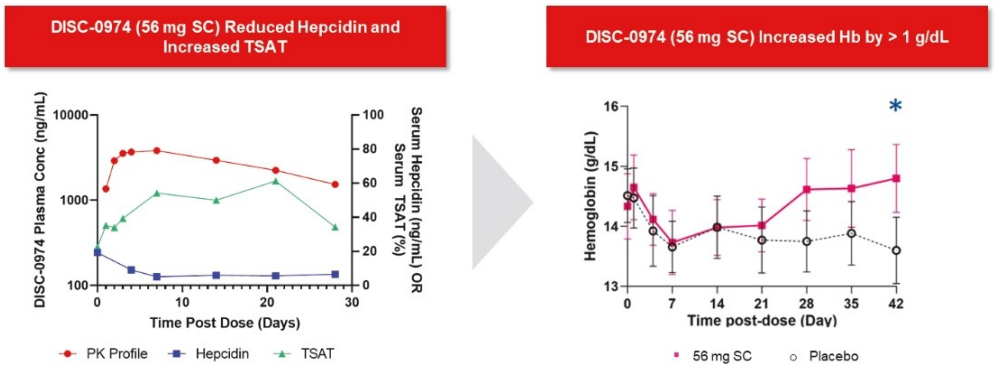
Specifically, in this Phase 1 study, a single dose of DISC-0974 resulted in rapid, dose-dependent and sustained decrease in serum hepcidin and a corresponding, robust increase in measures of circulating iron. This included more than a doubling of transferrin saturation from baseline at the highest dose level (56 mg SC). Changes in serum iron also corresponded with markers of iron mobilization and erythropoiesis, including decreased ferritin levels, increased reticulocyte hemoglobin, and increased mean corpuscular hemoglobin. These findings are consistent with the mechanism of action of DISC-0974.

DISC-0974 Phase 1 SAD Study in Healthy Volunteers: Effects on Hepcidin and Transferrin Saturation



Notably, at the 56 mg SC dose level, a single administration of DISC-0974 resulted in a statistically significant improvement in hemoglobin compared to placebo (+1.1 g/dL, p=0.009) at Day 42 and a marked increase in red blood cell count.

DISC-0974 Phase 1 SAD Study in Healthy Volunteers: Single 56 mg SC Dose Increases Hemoglobin



DISC-0974 was well-tolerated at all dose levels with no serious or severe adverse events, no adverse events leading to study withdrawal, and no adverse event greater than Grade 1. Plasma exposure was dose-related in the 14 to 56 mg SC range and effects were observed through 28 days post-dose, indicating a sustained and potentially clinically meaningful duration of action. These findings were presented at the 2022 EHA meeting in June 2022 and the 2022 ASH annual meeting.

Phase 1b / 2 Clinical Development Program in Anemia of Inflammation

Based on these findings, we initiated two Phase 1b/2 clinical trials of DISC-0974 in patients with anemia of different inflammatory diseases: a Phase 1b/2 clinical trial of DISC-0974 in patients with anemia of MF, which was initiated in June 2022, and a separate Phase 1b/2 clinical trial of DISC-0974 in patients with non-dialysis dependent CKD and anemia, which was initiated in February 2023. We presented interim data from both of these trials in December 2023 as well as additional interim data for anemia of MF in June 2024 and non-dialysis dependent CKD and anemia in October 2024, which included safety data and changes in hepcidin, iron, and hemoglobin levels for additional patients, as well as longer follow-up. In December 2024, we presented additional analyses of the Phase 1b study in anemia of MF and initiated a Phase 2 open-label clinical trial in patients with anemia of MF.

Ongoing Phase 1b/2 Clinical Trial in Patients with Anemia of Myelofibrosis

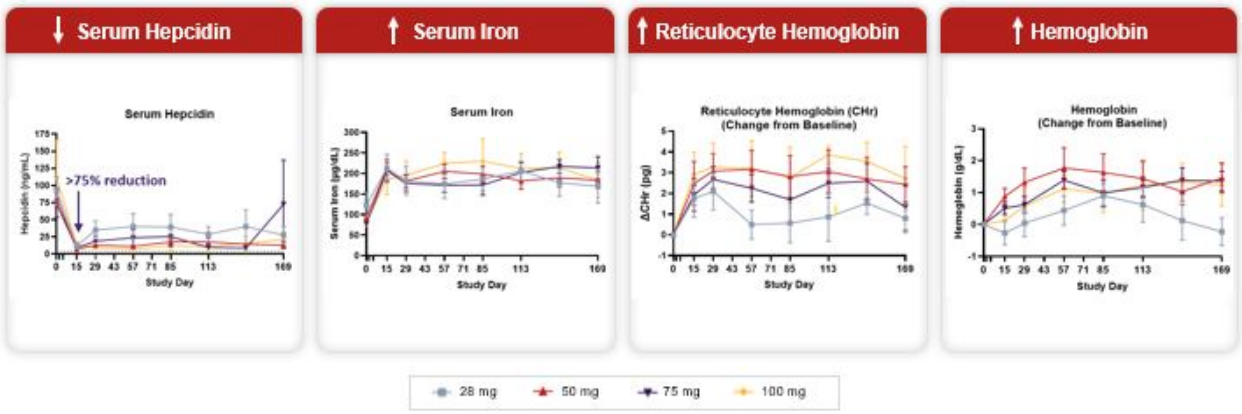
In June 2022, we initiated an open-label, multi-center, Phase 1b/2 trial to evaluate the safety, tolerability, and efficacy of DISC-0974 in patients with anemia of MF. The study endpoints include hepcidin levels, serum iron and markers of iron mobilization and measures of anemia benefit such as hemoglobin, reductions in transfusion burden and transfusion independence (TI) rate. The study allows enrollment of patients receiving stable background therapy, including JAK inhibitors. The study is being conducted in two parts:

- Phase 1b (Dose-Escalation): Ascending, monthly doses of DISC-0974 administered for six months to patients with anemia of MF, (Hb levels < 10 g/dL,), where a dose level is selected based on optimal increases in hemoglobin and serum iron;
- Phase 2 (Expansion Stage): Multiple doses of DISC-0974 administered once-a-month at the dose level selected from the Phase 1b portion of the study to patients with anemia of MF who are transfusion dependent or non-transfusion dependent, generally defined according to the baseline transfusion burden in a 12-week period.

Interim data from the Phase 1b part of this trial was first presented at the ASH annual meeting in December 2023, with additional interim data presented at the EHA annual meeting in June 2024. In December 2024, we presented additional analyses of this Phase 1b study at the ASH annual meeting. Of the 35 enrolled patients, data from 32 evaluable participants in the five dose cohorts (14 mg, 28 mg, 50 mg, 75 mg and 100 mg) were presented as of an October 17, 2024 data cutoff date. Two participants withdrew early from the trial due to an inadequate response, and two participants were not considered evaluable for efficacy due to incomplete transfusion data entry as of the October 17, 2024 data cutoff. Thirteen participants were taking concomitant JAK inhibitors. Twenty-two of the evaluable participants were non-transfusion dependent receiving no transfusions (nTD), five were transfusion dependent with one to two transfusions within a 12-week period at baseline (TD Low), and five were transfusion dependent with three to 12 transfusions within a 12-week period at baseline (TD High). DISC-0974 was administered subcutaneously at 14 mg (n=1), 28 mg (n=7), 50 mg (n=12), 75 mg (n=9), or 100 mg (n=6) every four weeks for up to six treatments; patients can remain on continuation treatment after the first six doses. Results demonstrated consistent, substantial decreases in hepcidin reaching >75% from baseline and corresponding increases in serum iron across patients, which translated to increased levels of reticulocyte hemoglobin and hemoglobin. 68% of baseline nTD patients achieved a hemoglobin increase of ≥1.5 g/dL during the study period and 50% had sustained mean increases for ≥12 weeks. 100% of TD Low patients achieved a ≥50% reduction

in transfusion requirement, and 80% of TD Low patients achieved transfusion independence, defined as no blood transfusions, over a 16-week period. 60% of TD High patients achieved a ≥50% reduction in transfusion requirement, and 40% of evaluable TD High patients achieved transfusion independence over a 12-week period. 54% of patients receiving concomitant JAK inhibitor therapy achieved a major hematologic response.

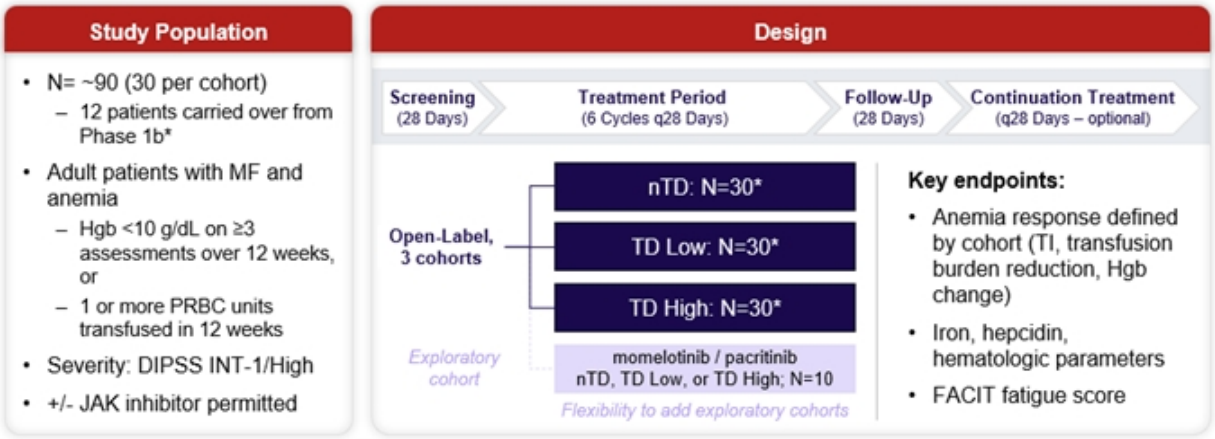
DISC-0974 Anemia of MF Phase 1b Clinical Trial Results: Pharmacodynamics



DISC-0974 was generally well-tolerated at all evaluated dose levels. Diarrhea was the only adverse event that was considered related to DISC-0974 and reported in two or more subjects. The majority of adverse events were not considered related to DISC-0974.

Ongoing Phase 2 Clinical Trial in Patients with Anemia of Myelofibrosis

In December 2024, we initiated the open-label Phase 2 part of our Phase 1b/2 clinical trial to evaluate the safety, tolerability and efficacy of DISC-0974 in patients with anemia of MF. The Phase 2 study design is summarized below.



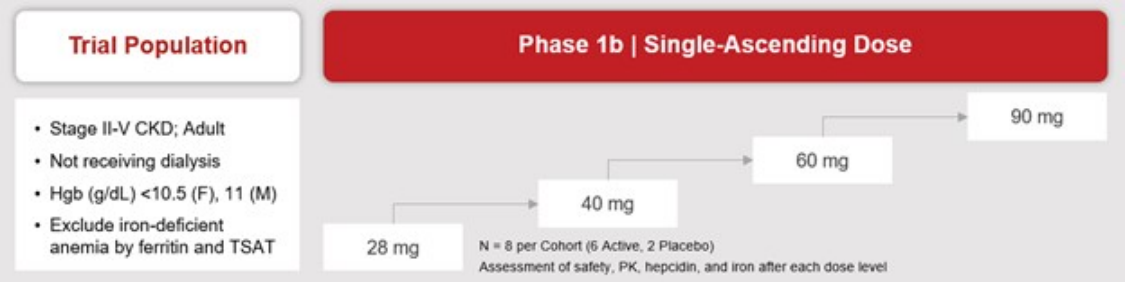
*Patients carried over from Phase 1b: nTD n=8, TD (Low + High) n=4

We expect to report initial data from this Phase 2 trial in the second half of 2025 and final data in 2026.

Ongoing Phase 1b/2 Clinical Trial in Patients with Non-Dialysis Dependent Chronic Kidney Disease (NDD-CKD)

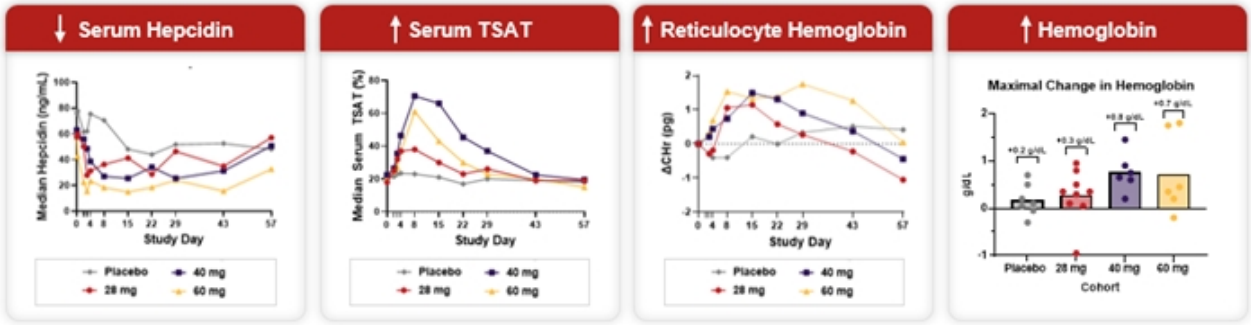
In February 2023, we initiated a Phase 1b/2 clinical trial to evaluate the safety, tolerability and efficacy of DISC-0974 in patients with CKD who are not receiving dialysis and are anemic. The study consists of two parts: a Phase 1b, randomized, placebo-controlled, single-ascending dose stage, where a dose level is selected based on optimal increases in serum iron; followed by an expansion stage where patients will receive multiple doses of DISC-0974 at the selected dose level. The study endpoints include hepcidin levels, serum iron and markers of iron mobilization and measures of anemia benefit such as hemoglobin.

Phase 1b Clinical Trial of DISC-0974 in NDD-CKD Patients with Anemia



Interim data from the Phase 1b part of this trial was first presented at the ASH annual meeting in December 2023, with additional interim data presented in October 2024. In the SAD portion of this trial, participants with Stage 2-5 non-dialysis dependent CKD were given a single dose of placebo (n=7) or DISC-0974 subcutaneously at 28 mg (n=9), 40 mg (n=6), or 60 mg (n=6). Dose escalation is ongoing in the SAD portion. This interim data set demonstrated substantial, durable, dose-dependent reduction in hepcidin from baseline compared to placebo across dose levels, with median reduction greater than 75% from baseline at highest dose level. There was meaningful and sustained increase in transferrin saturation from baseline compared to placebo across dose levels, with median increase up to three-fold from baseline at the highest dose level. In addition, there was early and sustained increase in mean reticulocyte hemoglobin from baseline across all dose groups through Day 22, with maximal mean values through Day 22 of +1.14 pg at 28 mg, +1.49 pg at 40 mg, and +1.53 pg at 60 mg, compared with +0.21 pg on placebo. Mean hemoglobin increased from baseline across dose groups over the study period, with changes greater than placebo as follows: +0.35 g/dL at 28 mg, +0.54 g/dL at 40 mg, and +0.55 g/dL at 60 mg. The mean maximal increase in hemoglobin was +0.8 g/dL at 40 mg and +0.7 g/dL at 60 mg compared with +0.2 g/dL on placebo, with a maximal observed individual increase in hemoglobin up to +0.95 g/dL at 28 mg, +1.5 g/dL at 40 mg, and +1.8 g/dL at 60 mg.

DISC-0974 Anemia of NDD-CKD Phase 1b Clinical Trial Interim Results: Hepcidin, Iron and Hemoglobin



DISC-0974 demonstrated acceptable safety and tolerability at all evaluated dose levels. The majority of adverse events were deemed not related to DISC-0974 and all adverse events assessed as treatment-related were Grade 1 or 2.

We expect to report data from the MAD portion of the trial in patients with non-dialysis dependent CKD and anemia in the second half of 2025. Subject to the results of such MAD portion of the study, we expect to initiate and report initial data from a Phase 2 clinical trial in patients with non-dialysis dependent CKD and anemia in 2026.

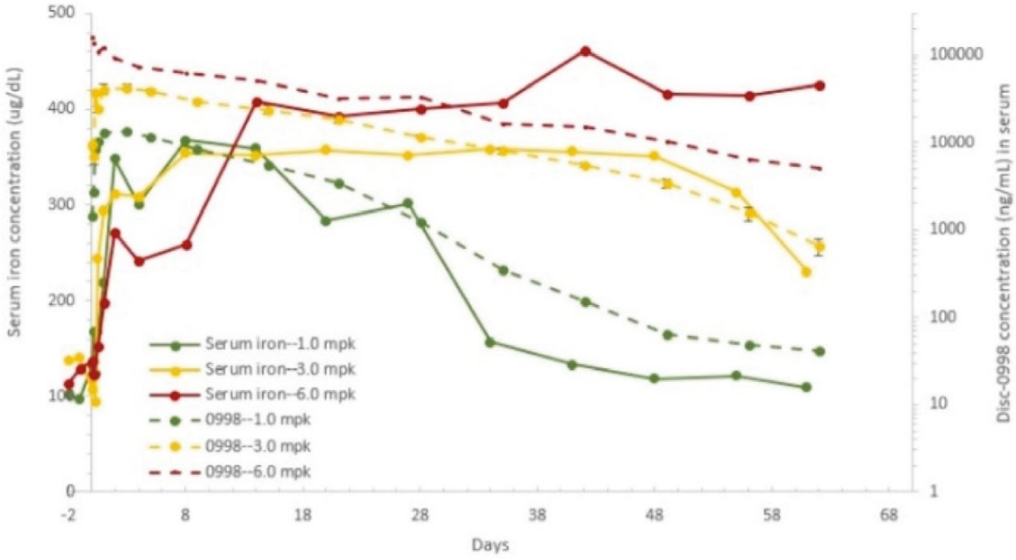
Our Second Hepcidin Suppression Program: DISC-0998

We are also developing a preclinical product candidate targeting hepcidin suppression, DISC-0998, an anti-HJV monoclonal antibody in-licensed from AbbVie. DISC-0998 is designed to be a highly selective anti-HJV mAb with an adapted Fc region to increase PK half-life. In preclinical studies DISC-0998 demonstrated biological activity, low immunogenicity potential, and desirable PK and PD properties.

A dose response PK/PD study of DISC-0998 in NHPs demonstrated that it had a lower clearance (~30 - 40%), higher volume of distribution (~30 - 70%), and longer half-life (~2 times), which translated to a longer duration of PD effects compared to DISC-0974. As shown below, a single dose of DISC-0998 resulted in sustained elevation of serum iron levels. If these data are confirmed

in humans, it would suggest the potential for an infrequent dosing regimen (such as potentially once every 2 or 3 months). We expect that such a dosing regimen would be perceived as convenient by patients and promote compliance.

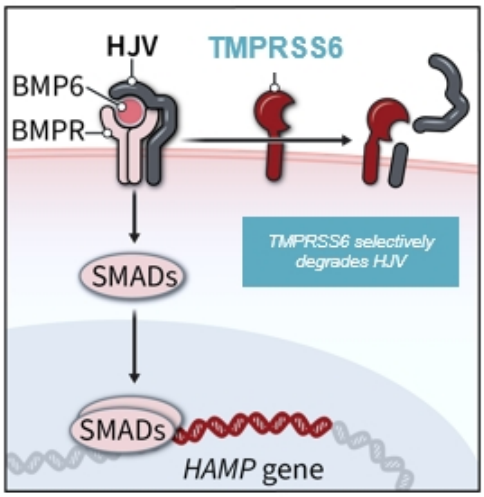
DISC-0998 Dose Response PK / PD Study (NHP)



Our Hepcidin Induction Program

We are also focused on developing product candidates designed to increase hepcidin levels and decrease serum iron levels to address a range of diseases where restricting iron would be beneficial, such as erythrocytosis of PV and diseases of iron overload, including HH, beta-thalassemia, and MDS. Our lead hepcidin induction program, DISC-3405, is a monoclonal antibody that is designed to target TMPRSS6. TMPRSS6 proteolytically degrades HJV in liver cells, as shown below. Inhibitors of TMPRSS6 are expected to increase HJV levels and thereby increase the expression of hepcidin. By inhibiting TMPRSS6, our compounds are designed to increase hepcidin production and, in turn, restrict iron availability. We selected TMPRSS6 as our target because the effects of reducing TMPRSS6 levels have been genetically confirmed in both animal knockout studies and in patients with iron-refractory iron deficiency anemia who lack fully functional genes encoding TMPRSS6.

TMPRSS6 Suppresses Hepcidin by Degrading HJV



Polycythemia Vera

PV is a chronic and rare myeloproliferative neoplasm characterized by the overproduction of red blood cells and increased red cell mass. It is frequently caused by acquired mutations of the JAK2 gene that drive abnormal proliferation of red blood cells. The increased number of red blood cells alters the viscosity of blood, causing it to thicken and placing patients at an increased risk of cardiovascular and thromboembolic events, such as heart attack and stroke. The prevalence of PV is estimated to be 44 to 57 cases per 100,000 persons, with approximately 150,000 patients with PV in the United States and with prevalence estimates in Europe ranging from 10 to 50 cases per 100,000 persons. PV tends to primarily affect individuals over 60 years old.

Current management of PV centers around depleting the number of red blood cells to maintain a patient's hematocrit (a measure of red blood cell mass) below 45%, the target threshold recommended by the National Comprehensive Cancer Network to reduce the risk of cardiovascular or thromboembolic events. Most patients receive low-dose aspirin and chronic therapeutic phlebotomy to physically remove blood and iron to limit erythropoiesis. However, most patients fail to achieve their target hematocrit levels and remain at risk for thrombosis and other complications. Moreover, phlebotomy causes discomfort and inconvenience for patients as well as side effects such as headaches, ringing in the ears, dizziness, and, over time, iron deficiency. Cytoreductive chemotherapy is recommended for patients at higher risk of thrombosis, including those who fail to meet their hematocrit threshold, or conversion to leukemia. These include hydroxyurea, interferons, or ruxolitinib, marketed as Jakafi, each of which are associated with side effects and can affect multiple cell types. There is currently no oral, non-cytoreductive option for the treatment of PV, which we believe would be beneficial for both low and high-risk patients.

Hereditary Hemochromatosis

HH is an inherited iron overload disorder caused by genetic mutations that lead to a deficiency in hepcidin production. This results in lifelong, abnormal iron homeostasis, specifically excessive absorption of iron from a patient's diet and dysregulated distribution of iron stores in the body. Over time, this leads to the accumulation of iron at toxic levels in multiple organs, including the liver, heart, joints, skin, and others, which, if left untreated, can lead to severe organ damage and potentially organ failure. HH is one of the most common genetic disorders among Whites, affecting millions worldwide, including over 1 million individuals in the United States alone.

There are currently no approved pharmacologic therapies for the treatment of HH and the standard of care is regular and lifelong therapeutic phlebotomy to deplete iron. However, similar to PV, phlebotomy can be a significant burden to patients due to discomfort, frequency of treatments required, and patient inconvenience. Additionally, despite not being approved for HH, iron chelators may be used off-label in certain cases but are often associated with toxicities, particularly with chronic use.

Other Iron Overload Disorders: Beta-Thalassemia and Myelodysplastic Syndromes

Iron overload is a serious and potentially fatal complication of blood disorders associated with ineffective erythropoiesis, such as beta-thalassemia or MDS. Patients with these conditions become severely anemic due to mutations that affect the production of functional red blood cells. This results in persistent and pathologic suppression of hepcidin, leading to unchecked increases in iron and, ultimately, accumulation of toxic iron levels in organs such as the heart, liver, and kidneys, as well as in the bone marrow, which exacerbates anemia.

Both beta-thalassemia and MDS arise from mutations that cause ineffective erythropoiesis. In the case of beta-thalassemia, the genetic defects are inherited and result in impaired synthesis of beta-globin chains, a critical subunit of hemoglobin. This deficiency results in the premature death of developing erythrocytes in the marrow or peripheral circulation, resulting in severe anemia. Globally, beta-thalassemia has an incidence of approximately 1 in 100,000 individuals, but can range significantly depending on the region. In Europe, where it is more common, beta-thalassemia has an incidence of 1 in 10,000 individuals, while it is rare in the United States, and exact numbers are not known. In contrast, MDS is a form of cancer where mutations prevent precursor cells in the marrow from maturing into functional erythrocytes, which results in severe anemia and other cytopenias. MDS tends to affect older patients and has an overall estimated annual incidence of 20-50 cases per 100,000 individuals over 60 years old. There are an estimated 60,000 to 170,000 patients with MDS in the United States and a similar number in Europe.

Currently, chronic red blood cell transfusions are a mainstay of treatment for anemia caused by beta-thalassemia and MDS. However, the benefit is transient and transfusions are burdensome and carry the risk of further iron overload. While iron chelation therapy may be used in conjunction, it requires careful dose titration and is often associated with toxicities. Recently, luspatercept (marketed as Reblozyl), a red blood cell maturation agent, was approved by the FDA and EMA to treat certain forms of beta-thalassemia and MDS, with a response rate of 21.4% and 37.9% for a primary endpoint of transfusion independence in the respective pivotal trials. A luspatercept label expansion study in the front-line setting of transfusion-dependent MDS showed a 58.5% response rate for transfusion independence with concurrent mean Hgb increase of ≥1.5 g/dL. Based on these response rates, many patients do not respond and would benefit from an alternative treatment. Lentiglobin, marketed as Zynteglo, and exagamglogene autoemcel, marketed as Casgevy, are gene therapies that were approved by the FDA and EMA for the treatment of a subset of patients with

beta-thalassemia requiring RBC transfusions, but uptake has been limited. Patients with more advanced forms of MDS may receive additional therapies such as lenalidomide, demethylating agents such as 5-azacitidine and decitabine, and chemotherapy.

Our Solution: DISC-3405 (MWTX-003), an anti-TMPRSS6 monoclonal antibody

In January 2023, we in-licensed MWTX-003 and other related molecules from Mabwell. In the Disc compound catalog, MWTX-003 is named DISC-3405. DISC-3405 is a monoclonal antibody designed to inhibit TMPRSS6, a serine protease that degrades HJV, a receptor required for hepcidin expression. TMPRSS6 plays a critical and specific function in iron metabolism by limiting the production of hepcidin. By inhibiting TMPRSS6, DISC-3405 has been shown in preclinical studies to increase endogenous production of hepcidin and reduce serum iron levels. This mechanism has been validated by human genetics, where patients with mutations in TMPRSS6 develop elevated hepcidin levels and an iron restrictive phenotype. In addition, iron restriction has been recently validated as a potential approach to treat PV. In a Phase 2 clinical trial conducted by a third-party, a peptide hepcidin mimetic administered weekly by subcutaneous injection lowered iron availability and reduced hematocrit in patients with PV, resulting in a substantial reduction in requirements for phlebotomy and improvements in disease symptoms. We are initially focused on developing our anti-TMPRSS6 programs as a potential treatment for PV with plans to expand development to encompass diseases of iron overload and other conditions where restriction of iron would have therapeutic benefit.

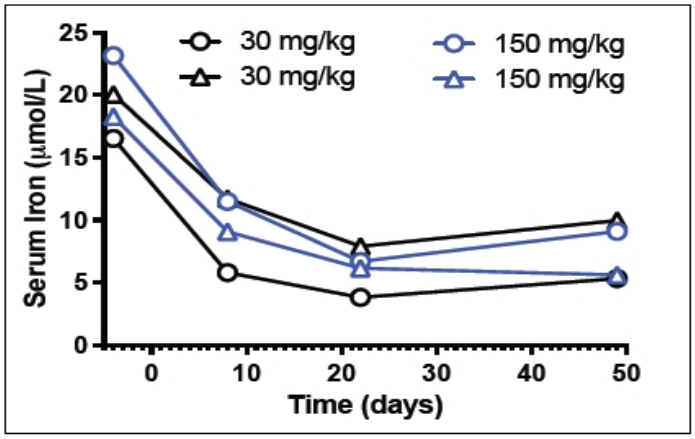
Preclinical Data

Preclinical studies of MWTX-003 have demonstrated that TMPRSS6 inhibition with a monoclonal antibody can induce hepcidin expression and consequently restrict iron availability. Specifically, preclinical studies have demonstrated:

- Dose-dependent induction of endogenous hepcidin production in rodent and NHP studies;
- Increases in hepcidin, reductions in serum and liver iron, and increases in red blood cells in a murine model of beta-thalassemia; and
- Reductions in iron and hematocrit in a murine model of PV.

Across several preclinical studies in both rodents and non-human primates, treatment with MWTX-003 was shown to increase hepcidin production and reduce serum iron levels. For example, in the below experiment in NHPs, treatment with a single, intravenously administered 30 mg/kg or 150 mg/kg dose of MWTX-003 resulted in deep and sustained suppression of serum iron levels for at least 3 weeks.

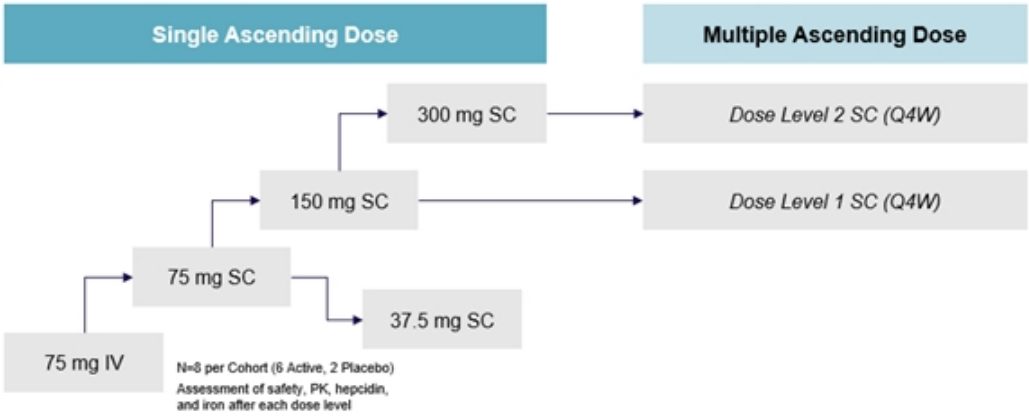
Treatment with MWTX-003 resulted in deep and sustained suppression of serum iron levels in normal non-human primates



The potential efficacy of MWTX-003 was also assessed in HbbTh3/+ mice, an established, genetic rodent model of beta-thalassemia. In this study, mice with mutations in the Hbb gene leading to underproduction of beta-globin genes were treated with 10 mg/kg MWTX-003 once every 3 days for 4 weeks which resulted in recapitulated features of beta-thalassemia such as ineffective erythropoiesis, iron overload and enlarged spleens. Treated mice showed decreased serum iron levels and liver non-heme iron, reduced splenomegaly and improved ineffective erythropoiesis compared to control. Additionally, a study was conducted assessing the efficacy of MWTX-003 in PV using a $Jak2^{V617F}$ bone marrow transplantation mouse model. In these mice, treatment with MWTX-003 at 2-10 mg/kg every 4 days for 3 weeks led to significantly increased serum hepcidin with reduced hematocrit levels, red blood cell counts, and hemoglobin concentration that were comparable to that of wildtype control mice.

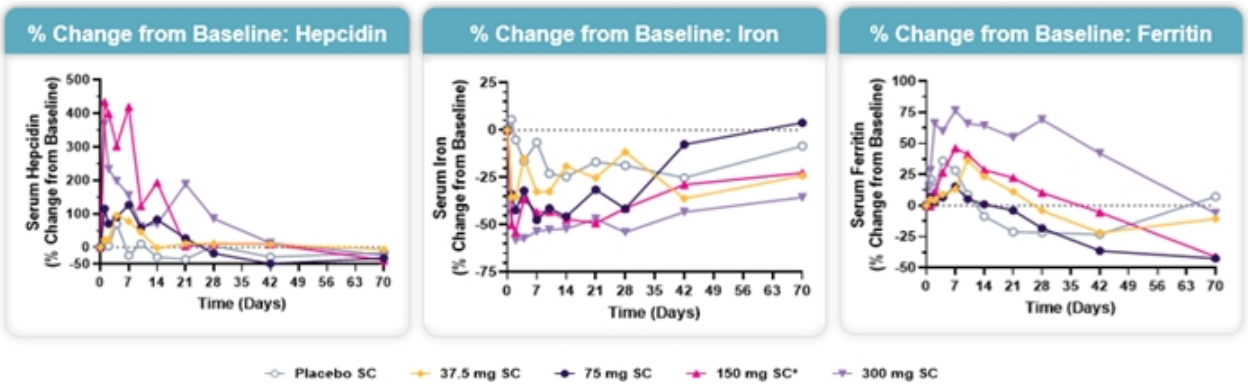
Phase 1 Clinical Trial

We initiated a Phase 1 clinical trial of DISC-3405 in healthy adult volunteers in October 2023. Interim data was presented from the SAD portion of the Phase 1 clinical trial in June 2024. We presented data from the MAD portion of the Phase 1 healthy volunteer study of DISC-3405 in December 2024. In the SAD portion of this trial, healthy males and females ages 18 to 65 were given a single dose of placebo (n=10) or DISC-3405 at 75 mg intravenously (IV) (n=6), 37.5 mg subcutaneously (SC) (n=6), 75 mg SC (n=6), 150 mg SC (n=6), or 300 mg SC (n=6). The MAD portion of this trial included placebo (n=4), 75 mg SC (n=6), and 150 mg SC (n=6) cohorts dosed every four weeks for a total of two doses.

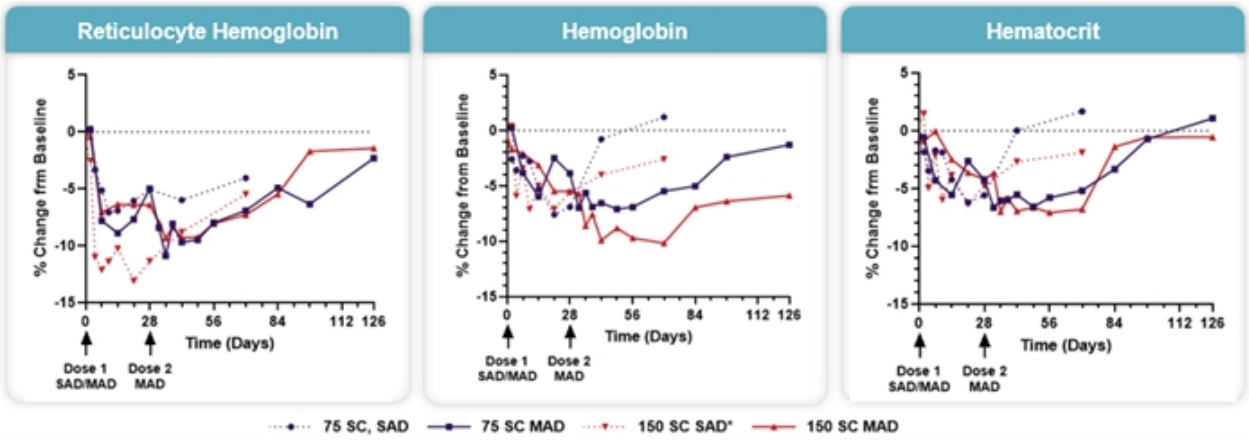


In this trial, DISC-3405 produced dose-related increases in serum hepcidin with corresponding reductions in serum iron across all dose levels. DISC-3405 also resulted in deep reductions in serum iron (ranging maximally from 50% to 80% from baseline) that were sustained and support a once-monthly SC dosing regimen. In addition, single and repeat dosing of DISC-3405 demonstrated meaningful reductions in hematologic parameters, including reticulocyte hemoglobin, hemoglobin, and hematocrit.

DISC-3405 Phase 1 Healthy Volunteers Study: Hepcidin, Iron and Ferritin



DISC-3405 Phase 1 Healthy Volunteers Study: Hematologic Response



DISC-3405 was generally well-tolerated at all evaluated dose levels, with no serious adverse events greater than Grade 2, or adverse events leading to study withdrawal.

Next Steps

We expect to develop DISC-3405 for the treatment of PV and other hematologic disorders, and plan to initiate a Phase 2 clinical trial of DISC-3405 in PV in the first half of 2025 and report data in 2026.

Manufacturing

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We rely on, and expect to continue to rely on for the foreseeable future, third-party contract development and manufacturing organizations, or CDMOs, to produce our product candidates and preclinical materials, including bitopertin, DISC-0974, DISC-0998, DISC-3405 and any candidates arising from our research programs, for preclinical and clinical use. We plan to continue to rely on third-party CDMOs for any future trials as well as for the commercial manufacture of our product candidates and preclinical materials, if approved. In addition, we contract with additional CDMOs to package, label, and distribute drug product for preclinical and clinical use.

Some of our product candidates are biological products. Manufacturing biologics can be particularly complex, especially in large quantities. Biologic products must be made consistently and in compliance with a clearly defined manufacturing process. We require that our CDMOs produce bulk drug substances and finished drug products in accordance with current Good Manufacturing Practices, or cGMPs, and all other applicable laws and regulations. We have assembled a team of experienced employees and external consultants to provide the required technical, quality, and regulatory oversight of our CDMOs and have implemented a comprehensive plan for regular audits of our CDMOs. We maintain agreements with our manufacturers that include confidentiality and intellectual property provisions to protect our proprietary rights related to our product candidates.

We obtain supplies of our product candidates from single-source CDMOs on a purchase order basis and do not currently have any long-term supply arrangements in place. While any reduction or halt in supply of our product candidates from these CDMOs could limit our ability to develop our product candidates until we find a qualified replacement CDMO, we have procured or are in the process of procuring sufficient supply to support our ongoing Phase 2 trials for bitopertin and DISC-0974, our ongoing Phase 1b trial for DISC-0974, our planned APOLLO post-marketing confirmatory trial for bitopertin, and our planned Phase 2 trial for DISC-3405. For example, to support our ongoing Phase 2 clinical trial of bitopertin, we have requalified, including establishing a shelf-life that would enable its use in clinical trials, Roche-manufactured drug substance and formulated it as film-coated tablets. To support APOLLO, our planned post-marketing confirmatory clinical trial of bitopertin, we have established the drug substance and drug product manufacturing process at a CDMO. In addition, we believe that we can identify and establish additional CDMOs to provide API and finished drug product without significant disruption to our business or clinical development timelines. As our pipeline programs expand and we build new process efficiencies, we expect to continually evaluate this strategy with the objective of satisfying demand for registration trials and, if approved, the manufacture, sale, and distribution of commercial products.

A commercial-scale production process has been designed for bitopertin, including a four-step chemical synthesis and an optimized oral formulation. To support the commercial launch of bitopertin, if approved, we are in the process of establishing a validated drug substance and drug product manufacturing process at a CDMO.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property. While we believe that our product candidates, preclinical programs, scientific capabilities, know-how, and experience provide us with competitive advantages, we compete in a highly competitive industry and face significant competition from many sources, including pharmaceutical and biotechnology companies, as well as academic institutions, governmental agencies, and private and public research institutions worldwide. Many of our competitors, either alone or through collaborations, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites, and recruiting patients in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. As a result, our competitors may discover, develop, license, or commercialize products before or more successfully than we do.

We face competition more specifically from companies that discover, develop, and market therapies for the treatment of hematologic diseases, including porphyrias and anemia associated with inflammatory diseases. There are many other companies, including large biotechnology and pharmaceutical companies, that have commercialized or are developing therapies for the same diseases that we are targeting with our product candidates. These companies include, but are not limited to, Agios Pharmaceuticals, Inc., Akebia Therapeutics, Inc., Amgen, Inc., Astellas Pharma, Inc., Bristol-Myers Squibb Company, FibroGen, Inc., GlaxoSmithKline, plc, Incyte Corporation, Ionis Pharmaceuticals, Inc., Keros Therapeutics, Inc., Merck & Co., Inc., Otsuka Pharmaceutical Co., Ltd. and Vifor Pharma AG, among others.

We are developing bitopertin, our lead product candidate in our heme biosynthesis modulation portfolio, for the treatment of EPs. If approved, bitopertin will face competition from melanocortin-1 receptor agonists, including afamelanotide, a subcutaneously implanted therapy that is approved in the U.S. and other territories and marketed as Scenesse by Clinuvel. Dersimelagon, an oral MC1R agonist, is currently in Phase 3 development by Mitsubishi Tanabe Pharma Corporation for the treatment of EPP or XLP. Portal Therapeutics, a subsidiary of BridgeBio Pharma, Inc., is currently testing in a first-in-human Phase 1 clinical study in healthy volunteers, PORT-77, an ABCG2 inhibitor, with a development goal to reduce plasma PPIX in EPP. In addition, there are other potential treatments currently in the discovery stages of development that may become competitors in the future. These therapies include, but are not limited to, gene therapies, heme biosynthesis modulators that target GlyT1 or other enzymes in the heme biosynthesis pathway, and molecules that target porphyrin export.

Bitopertin is a selective inhibitor of GlyT1 that we are developing to treat porphyrias and hematologic diseases. GlyT1 inhibition has been pursued in the past as an approach to treat schizophrenia. We are aware that Boehringer Ingelheim conducted a Phase 3 clinical study of BI 425809 (iclepertin), a GlyT1 inhibitor, for the improvement of cognition in patients with schizophrenia that failed to meet its primary endpoint. Other companies have also had research programs designed to inhibit GlyT1 as a treatment for schizophrenia, but to our knowledge, all of these have been discontinued at various stages of development. These include PF-03463275 (Pfizer Inc.), LY2365109 (Eli Lilly and Company), ORG25935 (Organon & Co.), ALX5407 (NPS Pharmaceuticals, Inc., now Shire plc), ASP2535 (Astellas Pharma Inc.) and others. We believe bitopertin has an optimal profile for development as a potential treatment for EP. However, we recognize that other companies may choose to develop a novel GlyT1 inhibitor or repurpose an existing one; if successfully developed as a treatment for EP, such a program would be a potential competitor to bitopertin.

We are also developing DISC-0974, our lead program in our hepcidin suppression portfolio, for the treatment of anemia caused by inflammatory diseases, including MF and CKD. For the treatment of anemia of MF, there are no approved therapies, but several classes of drugs are used off-label, including ESAs, such as Procrit (Janssen Pharmaceuticals, Inc.), Epogen and Aranesp (Amgen, Inc.), and Mircera (Roche), corticosteroids, and androgenic hormones, such as danazol. There are also multiple classes of drugs in development for the treatment of anemia. For example, multiple erythroid maturation agents are in development, such as luspatercept, which is in a myelofibrosis Phase 3 trial by Bristol-Myers Squibb, and KER-050, which is in a Phase 2 trial by Keros, Inc. In addition, GlaxoSmithKline has developed Ojaara (momelotinib), a JAK2 kinase inhibitor that lowers hepcidin by inhibiting ALK2 (a kinase that is similar to but less specific than HJV), which was approved to treat myelofibrosis patients with anemia.

For the treatment of non-dialysis dependent CKD and anemia, there are several therapies approved or in clinical development, including, but not limited to, ESAs, oral hypoxia inducible factor-prolyl hydroxylase inhibitors, or HIF-PHIs, which are approved in ex-U.S. territories but not in the U.S., and various forms of intravenous iron. We are not aware of any therapies in clinical development for the treatment of anemia of CKD that work by decreasing hepcidin levels. There are several therapies in development for the treatment of MF and CKD that do not directly target anemia, but their approvals may potentially change the treatment landscape and affect our ability to compete.

We are developing DISC-3405, an anti-TMPRSS6 monoclonal antibody designed to induce hepcidin production and restrict serum iron levels. There are several therapies in development that are also designed to increase hepcidin production or mimic hepcidin activity, such as hepcidin mimetics, TMPRSS6 inhibitors, and ferroportin inhibitors. These are in various stages of development by companies, including Regeneron, Silence Therapeutics plc, Ionis Pharmaceuticals, Inc., Rallybio, Protagonist

Therapeutics, Inc., Agios Pharmaceuticals, Inc., and CSL Vifor, among others. We may also face competition from therapies that are currently marketed or in development that affect pathways unrelated to hepcidin, including growth and differentiation factor-based therapies, cytoreductive therapies, and chemotherapeutic agents, among others.

We could see a reduction or elimination of our commercial opportunity if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient to administer, are less expensive, or receive a more favorable label than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for our products, which could result in our competitors establishing a strong market position before we are able to enter the market. The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the level of generic competition, and the availability of reimbursement from government and other third-party payors.

Collaborations and License Agreements

2019 Exclusive License Agreement with AbbVie Deutschland GmbH & Co. KG

In September 2019, we entered into an exclusive license agreement with AbbVie. Under the license agreement with AbbVie, or the AbbVie Agreement, we obtained an exclusive, worldwide license, with the right to sublicense to commercial pharmaceutical and biopharmaceutical companies (subject to AbbVie's prior consent or pre-authorization, except with respect to our affiliates), under certain patents and technical information of AbbVie, to make, have made, use, have used, sell, have sold, lease, have leased, import, have imported or otherwise transfer licensed products for all therapeutic, diagnostic and prophylactic uses in humans and animals, excluding uses in neuroscience and neurology. The anti-hemojuvelin antibodies, DISC-0974 and DISC-0998, are licensed products under the AbbVie Agreement. We are required to use commercially reasonable efforts to develop and commercialize at least one licensed product in certain major markets and to maximize net sales of licensed products in certain major markets.

Under the terms of the AbbVie Agreement, we made an initial license payment to AbbVie of $0.6 million. Additionally, we are required to pay certain development milestone payments for each licensed product, which milestone payments are up to $18.0 million in the aggregate, certain commercial milestone payments for each licensed product, which milestone payments are up to $45.0 million in the aggregate, and certain milestone payments based on the level of net sales of all licensed products worldwide, which milestone payments are up to $87.5 million in aggregate. In January 2025, we dosed the first patient in the Phase 2 clinical trial of DISC-0974. As a result, we made the first milestone payment of $3.0 million to AbbVie on January 31, 2025. We are also obligated to pay a royalty on net sales of licensed products at a low-single digit rate. The royalty rates are subject to up to a high first decile percentage reduction for lack of a valid claim on a country-by-country basis. See "Business-Intellectual Property-Iron Homeostasis Portfolio" for additional information concerning the intellectual property related to the AbbVie Agreement.

The obligation to pay royalties under the AbbVie Agreement expires on a licensed product-by-licensed product and country-by-country basis upon the later of expiry of (a) (i) the last valid claim of the licensed patents that cover such licensed product or the exploitation thereof in such country or (ii) the last-to-expire improvement patent in such country, whichever is later, (b) the expiration of regulatory exclusivity in such country, and (c) ten years from the first commercial sale of such product in such country.

The AbbVie Agreement expires upon expiry of the last remaining royalty obligation for the last licensed product. Under the AbbVie Agreement, either party may terminate the agreement upon the other party's uncured material breach or insolvency, and AbbVie may also terminate the agreement upon our failure to conduct any relevant material development or commercialization activity in a 12-month period, or, to the extent AbbVie is permitted pursuant to applicable law, a challenge by us of the licensed patents. We may terminate the agreement for any reason upon specified prior written notice to AbbVie.

In connection with the AbbVie Agreement, we also entered into a stock purchase agreement with AbbVie in September 2019, pursuant to which we agreed to issue 4,336,841 shares of our common stock to AbbVie, with 2,295,174 shares vesting immediately and 2,041,667 shares subject to a performance condition tied to the second and third subsequent closings of our Series A Preferred Stock financing. During the year ended December 31, 2020, the performance conditions were met and the remaining 2,041,667 shares vested. At the closing of the merger, shares of our common stock held before the merger were exchanged for shares of common stock in the combined publicly-traded company based on an exchange ratio of 0.1096.

2021 Exclusive License Agreement with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc.

In May 2021, we entered into a license agreement, or the Roche Agreement, with F. Hoffmann-La Roche Ltd. and Hoffmann-La Roche Inc., or together, Roche, pursuant to which Roche granted us an exclusive and sublicensable (subject to Roche's consent, not to be unreasonably withheld, except with respect to affiliates) worldwide license under certain of Roche's patent rights and know-how to develop and commercialize bitopertin, including certain backup compounds and derivatives, in all indications and for all therapeutic and prophylactic uses, except diagnostic use. Roche retained the rights with respect to diagnostic uses and its own internal non-clinical research purposes.

Under the Roche Agreement, Roche has an exclusive right to negotiate a license or purchase of all licensed compounds and products in certain specified circumstances. If we, for a specified period of time following entry into the Roche Agreement or before completion of a Phase 3 clinical trial of a licensed product (whichever is later), intend to enter into a sublicense or assignment of the Roche Agreement granting rights in the U.S., China or one or more major EU countries, then Roche will have a specified amount of time to perform diligence and negotiate the applicable license, purchase, or acquisition. If the parties are not able to come to terms during the applicable negotiation period, we are free to enter into the applicable transaction, provided that we may not enter into such a transaction on terms less favorable to us than the terms offered by Roche during a specified period after the conclusion of the negotiation period.

We are required to use commercially reasonable efforts to develop, seek regulatory approval and, on a country-by-country basis where such regulatory approval has been obtained, commercialize at least one licensed product in each such country.

Under the Roche Agreement, we paid Roche an initial license payment of $4.5 million and we will pay Roche up to an aggregate of $50.0 million in development and regulatory milestone payments for development and approval in a first indication, up to an aggregate of $35.0 million in development and regulatory milestone payments for development and approval in a second indication. The first potential milestones consist of a $10.0 million payment upon the initiation of the first Phase 3 clinical trial with a licensed product in a first indication, and a $15.0 million payment upon regulatory approval in the United States with a licensed product in a first indication. We will also pay Roche up to an aggregate of $120.0 million based on achievement of certain thresholds for annual net sales of licensed products. We are also obligated to pay a royalty on net sales of licensed products at a tiered rate ranging from the high-single digits to the high teens. The royalty rates are subject to a reduction (i) by 25% for lack of a valid claim covering the licensed product generating such sales, and (ii) by 50% for prevalence of generic products (or 25% if there are generic products on the market but there is still a valid claim), in each case on a country-by-country basis. Additionally, royalties are apportioned where licensed compounds are commercialized in combination products.

The obligation to pay royalties under the Roche Agreement expires on a licensed product-by-licensed product and country-by-country basis upon the later of (a) expiry of the last valid claim of the licensed and improvement patents that cover such licensed product in such country, (b) the expiration of regulatory exclusivity in such country, and (c) twelve years from the first commercial sale of such product in such country. The expiry of the last valid claim of the licensed and improvement patents subject to the Roche agreement is currently scheduled to occur in April 2035.

In connection with the Roche Agreement and pursuant to an addendum to the Roche Agreement between the parties executed in December 2021, we agreed to issue to Roche or its affiliates, immediately following the closing of the merger and for no additional consideration, shares of common stock estimated to be approximately 2.85% of the combined company's issued and outstanding capitalization immediately following the closing of our merger with Gemini Therapeutics, Inc., or Gemini, and our pre-closing financing. Upon completion of the merger, we issued 482,313 shares of common stock to Roche.

The Roche Agreement expires upon expiry of the last remaining royalty obligation for the last licensed product. Under the Roche Agreement, either party may terminate the agreement upon the other party's uncured material breach or insolvency. We may terminate the agreement for any reason upon specified prior written notice to Roche. In the event the Roche Agreement is terminated for certain causes, if Roche elects to continue development or commercialization of licensed products, certain single-digit royalties may be owed to us in connection with such continued development or commercialization.

2023 Exclusive License Agreement with Mabwell Therapeutics, Inc.

On January 19, 2023, we entered into an exclusive license agreement with Mabwell (a wholly-owned subsidiary of Mabwell (Shanghai) Bioscience Co., Ltd), or the Mabwell Agreement, pursuant to which Mabwell granted us an exclusive and sublicensable license under certain patent rights, know-how, and materials to develop and commercialize antibody products containing Mabwell's MWTX-001, MWTX-002, and MWTX-003 antibodies, along with limited variants thereof, in all fields of use, in all territories other than Greater China (Mainland China, Hong Kong, Macau and Taiwan) and Southeast Asia (Brunei, Myanmar, Cambodia, Timor-Leste, Indonesia, Laos, Malaysia, Philippines, Singapore, Thailand and Vietnam). We also granted Mabwell an exclusive, sublicensable, royalty-free license under our patents and know-how arising under the Mabwell Agreement to develop and commercialize licensed antibody products in Greater China and Southeast Asia.

Under the Mabwell Agreement, we paid an upfront payment of $10.0 million in March 2023. In addition, we are required to pay certain development and regulatory milestone payments for the licensed antibody products, for up to three indications, up to a maximum aggregate amount of $127.5 million, as well as certain commercial milestone payments for certain licensed antibody product net sales achievements, up to a maximum aggregate amount of $275.0 million. In October 2023, the first patient was dosed in the Phase 1 clinical trial in healthy volunteers of polycythemia vera for DISC-3405, resulting in a milestone payment of $5.0 million due to Mabwell. The next potential milestone payments include $10.0 million due upon the initiation of a Phase 2 clinical trial for DISC-3405 in a first indication and $5.0 million due upon the initiation of a Phase 1b clinical trial with a licensed product in a second indication. We are further obligated to pay a tiered percentage of revenue that we receive from our sublicensees (excluding revenue that is attributable to net sales on which royalty payments are due), ranging from a low third decile percentage

if the sublicense is granted prior to the initiation of a Phase 1 clinical trial of the licensed antibody product, to a low first decile percentage if the sublicense is granted after regulatory approval of the licensed antibody product. No sublicense revenue is due if the sublicense is granted after the first commercial sale of the licensed antibody product.

In addition, we are obligated to pay Mabwell a royalty on annual net sales of all licensed antibody products at a tiered rate ranging from low single-digits to high single-digits, subject to customary royalty reductions for (i) lack of a valid patent claim covering the licensed antibody product generating such sales, (ii) entry of a biosimilar product that equals or exceeds 20% of the total market share of the licensed antibody product, and (iii) a portion of any royalties paid to a third party for patents that claim the composition of matter or method of use of the licensed antibodies. Further, royalties are subject to customary apportionment calculations where the licensed antibodies are commercialized in combination products. The obligation to pay royalties under the Mabwell Agreement expires on a licensed antibody product-by-licensed antibody product and country-by-country basis upon the later of (a) expiration of the last valid patent claim of the licensed patents that cover such licensed antibody product in such country, (b) expiration of regulatory exclusivity for such licensed antibody product in such country, and (c) ten years from the first commercial sale of such licensed antibody product in such country.

Our license grant expires upon expiration of the last remaining royalty obligation for the last licensed antibody product in the last country. Either party may terminate the Mabwell Agreement prior to its expiration upon the other party's uncured material breach, insolvency, or a challenge of the validity or enforceability of the patents licensed to such other party under the agreement. We may also terminate the Mabwell Agreement for convenience on 60 days' written notice to Mabwell. In the event the Mabwell Agreement is terminated other than by us for cause, we have agreed to grant Mabwell an exclusive, sublicensable, worldwide license under our patent rights and know-how arising under the Mabwell Agreement to develop and commercialize products containing the licensed antibodies. In such circumstances, we and Mabwell will negotiate a royalty to be paid by Mabwell on the net sales of such products in the licensed territory.

Intellectual Property

Overview

We strive to protect the proprietary technology that we believe is important to our business, including seeking and maintaining patent protection in the United States and internationally for our current and future product candidates. We also rely on trademarks, copyrights, trade secrets, confidentiality procedures, employee disclosure, invention assignment agreements, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

We seek to obtain domestic and international patent protection, and endeavor to promptly file patent applications for new commercially valuable inventions. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

We plan to continue to expand our intellectual property estate by filing patent applications directed to pharmaceutical compositions, methods of treatment, methods of manufacture or identified from our ongoing development of our product candidates, which include both small molecule and biologic products, such as antibodies. Our success will depend on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce any patents that we may obtain, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties.

The patent positions of companies like ours are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent may be challenged in courts after issuance. Moreover, many jurisdictions permit third parties to challenge issued patents in administrative proceedings, which may result in further narrowing or even cancellation of patent claims. We cannot guarantee that our pending patent applications, or any patent applications that we may in the future file or license from third parties, will result in the issuance of patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or at all, whether the claims of any patent applications, should they issue, will cover our product candidates, or whether the claims of any issued patents will provide sufficient protection from competitors or otherwise provide any competitive advantage. We cannot predict the scope of claims that may be allowed or enforced in our patents. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Consequently, we may not obtain or maintain adequate patent protection for any of our product candidates.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months or potentially even longer, and because publication of discoveries in the scientific or patent literature often lags behind actual discoveries and patent application filings, we cannot be certain of the priority of inventions covered by pending patent applications. Accordingly, we may not have been the first to invent the subject matter disclosed in some of our patent applications or the first to file patent applications covering such subject matter, and we may have to participate in interference proceedings or derivation proceedings declared by the United States Patent and Trademark Office, or USPTO, to determine priority of invention. For more information regarding the risks related to our intellectual property, see "Risk Factors-Risks Related to Our Intellectual Property."

Patent Portfolio

Our patent portfolio includes patents and patent applications in the United States and selected jurisdictions outside of the United States. As of February 7, 2025, our patent portfolio in total consisted of 14 issued U.S. patents and 137 issued patents in foreign jurisdictions (e.g., Australia, China, United Kingdom, Germany, Mexico, Japan, and others), four PCT applications, 147 pending non-provisional applications (U.S., EP and other jurisdictions), and four pending U.S. provisional applications, which include claims directed to compositions and methods of use.

The patent portfolio includes patents and applications with claims related to the following programs:

Heme Biosynthesis Modulation Portfolio - GlyT1 Inhibition - Bitopertin

With regard to our heme biosynthesis modulation program, we own eight pending patent families directed to GlyT1 inhibitors (e.g., bitopertin) and various methods of treatment and use claims related, but not limited to EPP, XLP, CEP, DBA, PV, and hepatic porphyrias. Patents and pending applications directed to GlyT1 inhibitors (e.g., bitopertin) and methods of making and using them are expected to expire between 2041 and 2045, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. In particular, our first and second families are directed to methods of treating EPP, XLP, and CEP with bitopertin and related compounds, and solid forms of bitopertin, and these families, upon grant, will have a twenty-year statutory expiration date of 2041 and 2042, respectively. The first family has entered the national phase in the U.S. Australia, Canada, China, Europe, Hong Kong, Japan, and Korea. One U.S patent has granted from the first patent family and is directed to methods of treating EPP or XLP with bitopertin. This patent is expected to expire in 2042, without accounting for any potential terminal disclaimers, additional patent term adjustments or available extensions. The second family has entered the national phase in the U.S., Australia, Canada, China, Europe, and Japan. Our third family is directed to methods of treating polycythemias, including PV, with bitopertin and related compounds, and this family, upon grant, will have a twenty-year statutory expiration date of 2042, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. The third patent family has entered the national phase in the U.S. Australia, Brazil, Canada, China, Europe, Israel, Japan, Korea, and Mexico. Our fourth family is directed to methods of treating anemia associated with a ribosomal disorder (e.g., DBA) with bitopertin and related compounds, and this family, upon grant, will have a twenty-year statutory expiration date of 2042, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. The fourth patent family has entered the national phase in the U.S. Australia, Brazil, Canada, China, Europe, Israel, Japan, Korea, and Mexico. Our fifth family is directed to methods of treating hepatic porphyrias with bitopertin and related compounds, and this family, upon grant, will have a twenty-year statutory expiration date of 2043, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. The fifth patent family has entered the national phase in the U.S., Australia, Brazil, Canada, China, Europe, Israel, Japan, Korea, and Mexico. Our sixth family is directed to methods of treating EPP, XLP, and CEP with additional GlyT1 inhibitors, and this family, upon grant, will have a twenty-year statutory expiration date of 2042, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. The sixth patent family has entered the national phase in the U.S. Australia, Canada, China, Europe, and Japan. Our remaining two patent families are all directed to GlyT1 inhibitors (e.g., bitopertin) and methods of treating various disorders and conditions. These two patent families, upon grant, will have a twenty-year statutory expiration date from 2044 to 2045. We have also in-licensed multiple patent families from F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. comprising seven issued U.S. patents and additional granted patents in the following jurisdictions: Algeria, Australia, Austria, Belarus, Belgium, Brazil, Bulgaria, Canada, Chile, China, Croatia, Cyprus, Czech Republic, Denmark, Estonia, European Patent Convention, Finland, France, Germany, Great Britain, Greece, Gulf Cooperation Council, Hong Kong, Hungary, Indonesia, Ireland, Israel, Italy, Japan, Latvia, Lithuania, Luxembourg, Malaysia, Malta, Mexico, Monaco, Morocco, Netherlands, New Zealand, Norway, Philippines, Poland, Portugal, Republic of Korea, Republic of Serbia, Romania, Russian Federation, Singapore, Slovak Republic, Slovenia, South Africa, Spain, Sweden, Switzerland, Taiwan, Thailand, Turkey, Ukraine, and Vietnam. Patents and pending applications directed to bitopertin, synthetic intermediates, synthetic methods, synthetic processes of making bitopertin, treatment of hematologic disorders characterized by elevated cellular hemoglobin, and crystalline forms of bitopertin are expected to expire between 2025 and 2035, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. In particular, the first family is directed to composition of matter of bitopertin and processes of preparation. This family has an issued patent in the U.S. that expires in 2025. The second family is directed to processes of preparation of bitopertin, and this family has a twenty-year statutory expiration date of 2028. This family has issued patents in the U.S. and the following jurisdictions: Australia, Austria, Belgium, Brazil, Canada, China, European Patent Convention, Finland, France, Germany, Great Britain, Hungary, Ireland, Israel, Italy, Japan, Mexico, Netherlands, Republic of Korea, Spain, Sweden, and Switzerland. The third and fourth families are directed to synthetic processes for synthetic intermediates, and these families have twenty-year statutory expiration dates of 2026 and 2027, respectively. These families each have issued patents in the U.S. and the following jurisdictions: China, European Patent Convention, France, Germany, Great Britain, Japan, and Switzerland. The fifth family is directed to methods of treating hematological disorders characterized by elevated cellular hemoglobin levels with bitopertin, and this family has a twenty-year statutory expiration date of 2035. This family has issued patents in the U.S. and the following jurisdictions: Algeria, China, Croatia, Cyprus, European Patent Convention, France, Germany, Great Britain, Greece, Hong Kong, Indonesia, Italy, Japan, Malaysia, Morocco, Philippines, Portugal, Republic of Korea, Republic of Serbia, Slovenia, South Africa, Spain, Switzerland, and Turkey. The sixth family is directed composition of matter of additional GlyT1 inhibitors, and this family has a twenty-year statutory

expiration date of 2026. This family has issued patents in the U.S. and the following jurisdictions: China, European Patent Convention, France, Germany, Great Britain, Hong Kong, Japan, and Switzerland. The seventh family is directed to crystalline forms of bitopertin, and this family has a twenty-year statutory expiration date of 2027. This family has issued patents in the following jurisdictions: Australia, Austria, Belgium, Brazil, Bulgaria, Chile, Croatia, Cyprus, Czech Republic, Denmark, Estonia, European Patent Convention, Finland, France, Germany, Great Britain, Greece, Gulf Cooperation Council, Hungary, Indonesia, Ireland, Italy, Japan, Latvia, Lithuania, Luxembourg, Malaysia, Malta, Mexico, Monaco, Morocco, Netherlands, New Zealand, Norway, Philippines, Poland, Portugal, Republic of Korea, Republic of Serbia, Romania, Russian Federation, Singapore, Slovak Republic, Slovenia, South Africa, Spain, Sweden, Switzerland, Taiwan, Turkey, Ukraine, and Vietnam.

Several of the indications that we expect to pursue with bitopertin, including EPP, XLP and DBA, are rare diseases. We have received orphan drug designation from the FDA for bitopertin for the treatment of EPP and the European Committee for Orphan Medical Products adopted a positive opinion on Orphan Designation for bitopertin for treatment of EPP. We expect to file for an orphan drug designation for other indications in the United States and other relevant jurisdictions. If successful, orphan drug designation may provide a form of exclusivity for a period of years, described in greater detail below. See "Our Business-Governmental Regulation-Orphan Drug Designation and Exclusivity."

Iron Homeostasis Portfolio – Hepcidin Suppression – DISC-0974 and DISC-0998

With regard to our iron homeostasis portfolio, including our DISC-0974 and DISC-0998 programs, we own nine patent families, including one PCT patent application that has entered the national phase in Australia, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, and United States, one PCT patent application that has entered the national phase in Europe, and United States, one PCT patent application that has entered the national phase in Australia, Canada, China, Europe, Hong Kong, Israel, Japan, Korea, and United States, one PCT patent application that has entered the national phase in Australia, Brazil, Canada, China, Europe, Hong Kong, Israel, India, Japan, Korea, Mexico, New Zealand, Singapore, and United States, one PCT patent application that has entered the national phase in Australia, Brazil, Canada, China, Europe, Japan, Korea, Mexico, and United States, one PCT patent application that has entered the national phase in Australia, Brazil, Canada, China, Europe, Japan, Korea, Mexico, and United States, one pending PCT patent application, and two pending U.S. provisional applications. These patent families contain claims directed at composition of matter, method of treatment and use claims related to our initial indications, anemia of myelofibrosis and chronic kidney disease as well as other indications, e.g., anemia of inflammatory bowel disease and other anemias of chronic disease involving iron restriction from elevated hepcidin. Patents issuing from these applications are expected to expire between, 2040 and 2045 without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. Further, several of the above owned patent applications within our iron homeostasis portfolio are Joint Patents according to the AbbVie Agreement, whereby we own the patent applications and any patents granted thereon jointly with AbbVie, and we hold an exclusive license to AbbVie's interest in the patent applications and any patents granted thereon pursuant to the AbbVie Agreement.

We also in-license a patent family from AbbVie comprised of four issued U.S. patents that are expected to expire in 2032 and 2035, and issued patents in Australia, Canada, Brazil, China, the United Kingdom, Germany, Mexico, and Japan that are each expected to expire in 2032. These in-licensed patents include composition of matter claims, as well as method of treatment and use claims related to diseases of iron metabolism, such as anemia of chronic disease, iron-refractory iron-deficiency anemia, and anemia of chronic kidney disease. This in-licensed patent family also includes seven pending non-provisional applications in the United States, Australia, Brazil, Canada, China, Europe, and Japan. Any patents that issue on these pending non-provisional applications are likewise expected to expire in 2032, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions.

Iron Homeostasis Portfolio – Hepcidin Induction – MWTX-001, MWTX-002, and DISC-3405 (formerly MWTX-003)

With regard to our TMPRSS6 inhibitor program, we in-license a patent family from Mabwell comprised of nine pending non-provisional applications in Australia, Canada, Europe, India, Japan, Korea, and United States; and one pending PCT application. These in-licensed patent applications include composition of matter claims to antibodies designed to inhibit TMPRSS6, including MWTX-001, MWTX-002, and DISC-3405, as well as method of treatment and use claims related to diseases of iron metabolism (e.g., PV). Two U.S patents have been granted from the in-licensed patent family and are directed to the DISC-3405 TMPRSS antibody. These patents are expected to expire in 2041, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. We also own one pending PCT application directed to methods of treatment claims related to diseases of iron metabolism. This application, upon grant, will have a twenty-year statutory expiration date of 2044, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. We also own one provisional patent application directed to TMPRSS6 antibody formulations. This application, upon grant, will have a twenty-year statutory expiration date of 2046. Further, several of the above owned patent applications within our iron homeostasis portfolio are Joint Improvement Patents according to the Mabwell Agreement, whereby we own the patent applications and any patents granted thereon jointly with Mabwell, and we hold an exclusive license to Mabwell's interest in the patent applications and any patents granted thereon pursuant to the Mabwell Agreement.

We also own a first patent family comprising one patent in India and seven pending non-provisional applications in U.S., Europe, Japan, Australia, Canada, and China, and a second and third patent family each comprising one non-provisional application in the U.S. These patent families are directed to compounds that inhibit TMPRSS6 and methods of using the same. Any patents that issue on the non-provisional applications in the first family are expected to expire in 2039, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions. Any patents that issue from the non-provisional application in the second or third families are expected to expire in 2041 and 2044, respectively, without accounting for any potential terminal disclaimers, available patent term adjustments or extensions.

Patent Term

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, including the U.S., the base term is 20 years from the filing date of the earliest-filed non-provisional patent application from which the patent claims priority. The term of a U.S. patent can be lengthened by patent term adjustment, which compensates the owner of the patent for administrative delays at the USPTO. In some cases, the term of a U.S. patent is shortened by terminal disclaimer that reduces its term to that of an earlier-expiring patent. The term of a U.S. patent may be eligible for patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act, to account for at least some of the time the drug is under development and regulatory review after the patent is granted. With regard to a drug for which FDA approval is the first permitted marketing of the active ingredient, the Hatch-Waxman Act allows for extension of the term of one U.S. patent that includes at least one claim covering the composition of matter of such an FDA-approved drug, an FDA-approved method of treatment using the drug and/or a method of manufacturing the FDA-approved drug. The extended patent term cannot exceed the shorter of five years beyond the non-extended expiration of the patent or fourteen years from the date of the FDA approval of the drug, and a patent cannot be extended more than once or for more than a single product. During the period of extension, if granted, the scope of exclusivity is limited to the approved product for approved uses. Some foreign jurisdictions, including Europe and Japan, have analogous patent term extension provisions, which allow for extension of the term of a patent that covers a drug approved by the applicable foreign regulatory agency.

In the future, if and when our product candidates receive FDA approval, we expect to apply, if appropriate, for patent term extension on patents directed to those product candidates, their methods of use and/or methods of manufacture. However, there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions. For more information regarding the risks related to our intellectual property, see "Risk Factors-Risks Related to Our Intellectual Property."

Trade Secrets

In addition to patents, we rely on trade secrets and know-how to develop and maintain our competitive position. We typically rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. We protect trade secrets and know-how by establishing confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements provide that all confidential information developed or made known during the course of an individual or entities' relationship with us must be kept confidential during and after the relationship. These agreements also provide that all inventions resulting from work performed for us or relating to our business and conceived or completed during the period of employment or assignment, as applicable, shall be our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary information by third parties.

Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. For more information regarding the risks related to our intellectual property, see "Risk Factors-Risks Related to Our Intellectual Property."

Governmental Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, recordkeeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of drugs and biologics. We, along with our vendors, contract research organizations, or CROs, clinical investigators and contract manufacturing organizations, or CMOs, will be required to navigate the various preclinical, clinical, manufacturing and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval of our product candidates. The process of obtaining regulatory approvals of drugs and biologics and ensuring subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources.

In the United States, the FDA regulates drug products under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and biologics under the FD&C Act and the Public Health Service Act, or PHSA, as amended, and their implementing regulations. Both drugs and biologics are also subject to other federal, state and local statutes and regulations. We believe that bitopertin, which is a small molecule, will be regulated by the FDA as a drug product, and DISC-0974, DISC-0998 and DISC-3405, which are monoclonal antibodies, will be regulated by FDA as biologic products. If we fail to comply with applicable FDA or other requirements at any time with respect to product development, clinical testing, approval or any other regulatory requirements relating to product manufacture, processing, handling, storage, quality control, safety, marketing, advertising, promotion, packaging, labeling, export, import, distribution, or sale, we may become subject to administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, issuance of clinical holds for ongoing studies, suspension or revocation of approved applications, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution.

Our product candidates must be approved for therapeutic indications by the FDA before they may be marketed in the United States. For drug product candidates regulated under the FD&C Act, FDA must approve an NDA. For biologic product candidates regulated under the FD&C Act and PHSA, FDA must approve a Biologics License Application, or BLA. The process is similar for both drugs and biologics and generally involves the following:

- completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements;
- completion of the manufacture, under current Good Manufacturing Practices, or cGMP, conditions, of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;
- submission to the FDA of an IND which must become effective before clinical trials may begin and must be updated annually and when certain changes are made;
- approval by an institutional review board, or IRB, or independent ethics committee at each clinical trial site before each trial may be initiated;
- performance of adequate and well-controlled clinical trials in accordance with applicable IND regulations, good clinical practice, or GCP, requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- preparation and submission to the FDA of an NDA or BLA;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to file the application for review;
- satisfactory completion of one or more FDA pre-approval or pre-license inspections of the manufacturing facility or facilities where the drug will be produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug or biological product's identity, strength, quality and purity;
- satisfactory completion of FDA audit of the clinical trial sites that generated the data in support of the NDA or BLA;
- payment of user fees for FDA review of the NDA or BLA, unless a waiver is applicable; and
- FDA review and approval of the NDA or BLA, including, where applicable, consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug in the United States.

Preclinical Studies and Clinical Trials for Drugs and Biologics

Before testing any drug or biologic in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluations of product chemistry, formulation and stability, as well as *in vitro* and animal studies to assess safety and in some cases to establish the rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulation and requirements, including GLP requirements for safety/toxicology studies. The results of the preclinical studies, together with manufacturing information and analytical data, must be submitted to the FDA as part of an IND.

An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before clinical trials may begin. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes the results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. Some long-term preclinical testing may continue after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the clinical trial, including concerns that

human research subjects will be exposed to unreasonable health risks, and imposes a full or partial clinical hold. FDA must notify the sponsor of the grounds for the hold and any identified deficiencies must be resolved before the clinical trial can begin. Submission of an IND may result in the FDA not allowing clinical trials to commence or not allowing clinical trials to commence on the terms originally specified in the IND. A clinical hold can also be imposed once a trial has already begun, thereby halting the trial until the deficiencies articulated by FDA are corrected.

The clinical stage of development involves the administration of the product candidate to healthy volunteers or patients under the supervision of qualified investigators, who generally are physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirements that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters and criteria to be used in monitoring safety and evaluating effectiveness. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable compared to the anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. The FDA, the IRB, or the sponsor may suspend or discontinue a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trials to public registries. Information about clinical trials, including results for clinical trials other than Phase 1 investigations, must be submitted within specific timeframes for publication on www.ClinicalTrials.gov, a clinical trials database maintained by the National Institutes of Health.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a clinical trial may move forward at designated check points based on access that only the group maintains to available data from the trial and may recommend halting the clinical trial if it determines that the participants or patients are being exposed to an unacceptable health risk or other grounds, such as no demonstration of efficacy. Other reasons for suspension or termination may be made by us based on evolving business objectives and/or competitive climate.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, FDA will nevertheless accept the results of the study in support of an NDA or BLA if the study was well-designed and well-conducted in accordance with GCP requirements, including that the clinical trial was performed by a qualified investigator(s); the data are applicable to the U.S. population and U.S. medical practice; and the FDA is able to validate the data through an onsite inspection if deemed necessary.

Clinical trials to evaluate therapeutic indications to support NDAs and BLAs for marketing approval are typically conducted in three sequential phases, which may overlap.

- *Phase 1* - Phase 1 clinical trials involve initial introduction of the investigational product in a limited population of healthy human volunteers or patients with the target disease or condition. These studies are typically designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, excretion the side effects associated with increasing doses, and, if possible, to gain early evidence of effectiveness.
- *Phase 2* - Phase 2 clinical trials typically involve administration of the investigational product to a limited patient population with a specified disease or condition to evaluate the drug's potential efficacy, to determine the optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.
- *Phase 3* - Phase 3 clinical trials typically involve administration of the investigational product to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval and physician labeling. Generally, two adequate and well-controlled Phase 3 trials are required by the FDA for approval of an NDA or BLA.

Post-approval trials, sometimes referred to as Phase 4 clinical trials or post-marketing studies, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of NDA or BLA approval.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA. Written IND safety reports must be submitted to the FDA and the investigators fifteen days after the trial sponsor determines the information qualifies for reporting for serious and unexpected suspected adverse events, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human volunteers and any clinically important increase in the rate of a serious

suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must also notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction as soon as possible but in no case later than seven calendar days after the sponsor's initial receipt of the information. During the development of a new drug or biological product, sponsors have the opportunity to meet with the FDA at certain points, including prior to submission of an IND, at the end of Phase 2 and before submission of an NDA or BLA. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date and for the FDA to provide advice on the next phase of development.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. For biological products in particular, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined in order to help ensure safety, purity and potency. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Marketing Approval for Drugs and Biologics

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA or BLA requesting approval to market the product for one or more indications. An NDA is a request for approval to market a new drug for one or more specified indications and must contain proof of the drug's safety and efficacy for the requested indications. A BLA is a request for approval to market a new biologic for one or more specified indications and must contain proof of the biologic's safety, purity and potency for the requested indications. The marketing application is required to include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational drug, or the safety, purity and potency of the investigational biologic, to the satisfaction of the FDA. FDA must approve an NDA or BLA before a drug or biologic may be marketed in the United States. The FDA reviews all submitted NDAs and BLAs to ensure they are sufficiently complete to permit substantive review before it accepts them for filing and may request additional information rather than accepting the NDA or BLA for filing. The FDA must make a decision on accepting an NDA or BLA for filing within 60 days of receipt, and such decision could include a refusal to file by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the NDA or BLA. The FDA reviews an NDA or BLA to determine, among other things, whether the product is safe and effective for the indications sought and whether the facility in which it is manufactured, processed, packaged or held meets standards, including cGMP requirements, designed to assure and preserve the product's continued identity, strength, quality and purity. Under the goals and polices agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA targets ten months, from the filing date, in which to complete its initial review of a new molecular entity NDA or BLA and respond to the applicant, and six months from the filing date of a new molecular entity NDA or BLA for priority review. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs or BLAs, and the review process is often extended by FDA requests for additional information or clarification.

Further, under PDUFA, as amended, each NDA or BLA must be accompanied by a substantial user fee. The FDA adjusts the PDUFA user fees on an annual basis. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs or BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA also may require submission of a Risk Evaluation and Mitigation Strategy, or REMS, if it believes that a risk evaluation and mitigation strategy is necessary to ensure that the benefits of the drug outweigh its risks. A REMS can include use of risk evaluation and mitigation strategies like medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, special monitoring or other risk-minimization tools.

The FDA may refer an application for a novel drug or biologic to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA or BLA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. Additionally,

before approving an NDA or BLA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP and other requirements and the integrity of the clinical data submitted to the FDA.

After evaluating the NDA or BLA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA or BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response Letter without first conducting required inspections, testing submitted product lots, and/or reviewing proposed labeling. In issuing the Complete Response Letter, the FDA may require additional clinical or preclinical testing or recommend other actions, such as requests for additional information or clarification, that the applicant might take in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications.

Even if the FDA approves a product, depending on the specific risk(s) to be addressed it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition with either a patient population of fewer than 200,000 individuals in the United States, or a patient population of 200,000 or more individuals in the United States when there is no reasonable expectation that the cost of developing and making the product available in the United States for the disease or condition will be recovered from sales of the product. Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, though companies developing orphan products are eligible for certain incentives, including tax credits for qualified clinical testing and waiver of application fees.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same indication, except in limited circumstances, such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the indication for which the orphan product has exclusivity or obtain approval for the same therapeutic agent for a different indication than that for which the orphan product has exclusivity. Orphan product exclusivity could block the approval of one of our products for seven years if a competitor obtains approval for the same therapeutic agent for the same indication before we do, unless we are able to demonstrate that our product is clinically superior. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Further, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

The FDA may further reevaluate its regulations and policies under the Orphan Drug Act. It is unclear as to how, if at all, the FDA may change the orphan drug regulations and policies in the future.

Rare Pediatric Disease Designation and Priority Review Vouchers

Under the FD&C Act, the FDA incentivizes the development of products that meet the definition of a "rare pediatric disease," defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects 200,000 or more in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent

human drug application after the date of approval of the rare pediatric disease drug product, referred to as a priority review voucher, or PRV. A sponsor may request rare pediatric disease designation from the FDA prior to the submission of its NDA or BLA. A rare pediatric disease designation does not guarantee that a sponsor will receive a PRV upon approval of its NDA or BLA. Moreover, a sponsor who chooses not to submit a rare pediatric disease designation request may nonetheless receive a PRV upon approval of its marketing application if it requests such a voucher in its original marketing application and meets all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Under current statutory provisions, the FDA may award a priority review voucher for an approved rare pediatric disease product application only if the sponsor has received rare pediatric disease designation for the drug by December 20, 2024, and after September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. Congress may vote to reauthorize this program, but its future remains uncertain.

Expedited Development and Review Programs for Drugs and Biologics

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs and biologics to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. These programs include fast track designation, breakthrough therapy designation, priority review and accelerated approval, and the purpose of these programs is to either expedite the development or review of important new drugs and biologics to get them to patients more quickly than standard FDA review timelines typically permit.

A new drug or biologic is eligible for fast track designation if it is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address unmet medical needs for such disease or condition. Fast track designation applies to the combination of the product candidate and the specific indication for which it is being studied. Fast track designation provides increased opportunities for sponsor interactions with the FDA during preclinical and clinical development, in addition to the potential for rolling review once a marketing application is filed. Rolling review means that the FDA may review portions of the marketing application before the sponsor submits the complete application. Additionally, the FDA may rescind a fast track designation if it believes that the designation is no longer supported by data emerging in the clinical trial process.

In addition, a new drug or biologic may be eligible for breakthrough therapy designation if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug or biologic, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Breakthrough therapy designation provides all the features of fast track designation in addition to intensive guidance on an efficient product development program beginning as early as Phase 1, and FDA organizational commitment to expedited development, including involvement of senior managers and experienced review staff in a cross-disciplinary review, where appropriate.

Any product submitted to the FDA for approval, including a product with fast track or breakthrough therapy designation, may also be eligible for additional FDA programs intended to expedite the review and approval process, including priority review designation and accelerated approval. A product is eligible for priority review, once an NDA or BLA is submitted, if the product that is the subject of the marketing application has the potential to provide a significant improvement in safety or effectiveness in the treatment, diagnosis or prevention of a serious disease or condition. Under priority review, the FDA's goal date to take action on the marketing application is six months compared to ten months for a standard review.

Products are eligible for accelerated approval if they can be shown to have an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or an effect on a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, which is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Accelerated approval is generally contingent on a sponsor's agreement to conduct, in a diligent manner, adequate and well-controlled additional post-approval confirmatory trials to verify and describe the product's clinical benefit. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is now permitted to require that such trials be underway prior to approval or within a specific time period after the date accelerated approval is granted. Additionally, under FDORA, the FDA has increased authority for expedited procedures to withdraw approval of a product or an indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, for products being considered for accelerated approval, the FDA generally requires, unless otherwise informed by the agency, that all advertising and promotional materials intended for dissemination or publication within 120 days of marketing approval be submitted to the agency for review during the pre-approval review period. After the 120-day period has passed, all advertising and promotional materials must be submitted at least 30 days prior to the intended time of initial dissemination or publication.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or the time period for FDA review or approval may not be shortened. Furthermore, fast track designation, breakthrough therapy designation, priority review and accelerated approval do not change the scientific or medical standards for approval or the quality of evidence necessary to support approval, though they may expedite the development or review process.

Pediatric Information and Pediatric Exclusivity

Under the Pediatric Research Equity Act, or PREA, as amended, certain NDAs and BLAs and certain NDA and BLA supplements must contain data that can be used to assess the safety and efficacy of the product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. The FD&C Act requires that a sponsor who is planning to submit a marketing application for a product candidate that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs. Unless otherwise required by regulation, PREA does not apply to a drug or biologic for an indication for which orphan designation has been granted.

A product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods for biologics and drugs and patent terms for drugs. This six-month exclusivity, which runs from the end of other exclusivity protection for biologics and drugs or patent term for drugs, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

U.S. Post-Approval Requirements for Drugs and Biologics

Drugs and biologics manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, reporting of adverse experiences with the product, complying with promotion and advertising requirements, which include restrictions on promoting products for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities.

Although physicians may prescribe approved products for off-label uses, manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, including not only by company employees but also by agents of the company or those speaking on the company's behalf, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including investigation by federal and state authorities. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Promotional materials for approved drugs and biologics must be submitted to the FDA in conjunction with their first use or first publication. Further, if there are any modifications to the drug or biologic, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA or BLA or NDA or BLA supplement, which may require the development of additional data or preclinical studies and clinical trials.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA or BLA. For example, the FDA may require post-market testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. In addition, manufacturers and their subcontractors involved in the manufacture and distribution of approved drugs and biologics are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMPs, which impose certain procedural and documentation requirements on sponsors and their CMOs. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that a sponsor may use. Additionally, manufacturers and other parties involved in the drug supply chain for prescription drug and biological products must also comply with product tracking and tracing requirements and for notifying FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, manufacturers must continue to expend time money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance. Failure to comply with statutory and regulatory requirements may subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, product seizures, injunctions, civil penalties or criminal prosecution. There is also a continuing, annual program user fee for any marketed product.

The FDA may withdraw approval of a product if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, requirements for post-market studies

or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs; and
- mandated modification of promotional materials and labeling and issuance of corrective information

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of our future product candidates, some of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit restoration of the patent term of up to five years as compensation for patent term lost during the FDA regulatory review process. Patent term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or BLA plus the time between the submission date of an NDA or BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond a patent's current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA or BLA.

Marketing exclusivity provisions under the FDCA also can delay the submission or the approval of certain drug product applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an Abbreviated New Drug Application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

U.S. Biosimilars and Exclusivity

The Biologics Price Competition and Innovation Act, or BPCIA, created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars in the United States. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual,

the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed.

During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

Other Regulatory Matters

Manufacturing, labeling, packaging, distribution, sales, promotion and other activities of product candidates following product approval, where applicable, or commercialization are also potentially subject to federal and state consumer protection and unfair competition laws, among other requirements to which we may be subject. Additionally, the activities associated with the commercialization of product candidates is subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, which may include the Centers for Medicare & Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments and governmental agencies.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive recordkeeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements may subject firms to legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, relabeling or repackaging, or refusal to allow a firm to enter into supply contracts, including government contracts. Any claim or action against us for violation of these laws, even if we successfully defends against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on marketing, sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in statutes, regulations, or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling or packaging; (iii) the recall or discontinuation of our products; or (iv) additional recordkeeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Other Healthcare Laws

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business that may constrain the financial arrangements and relationships through which we research, as well as sell, market and distribute any products for which we obtain marketing authorization. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, and transparency laws and regulations related to drug pricing and payments and other transfers of value made to physicians and other healthcare providers. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of operations, integrity oversight and reporting obligations, exclusion from participation in federal and state healthcare programs and responsible individuals may be subject to imprisonment.

Healthcare Reform Measures

Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, among other things, subjected biologic products to potential competition by lower-cost biosimilars; increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed

care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; created a Medicare Part D coverage gap discount program, under which manufacturers were required to agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D (later revised by the Inflation Reduction Act); and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment, there have been numerous judicial, administrative, executive, and legislative challenges to certain aspects of the ACA. On June 17, 2021, for example, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. In addition, multiple executive orders have been issued (and we anticipate that additional executive orders will be issued under the current administration) that have sought to reduce prescription drug costs. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the current administration may reverse or otherwise change these measures, both the current administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs. It is unclear how these healthcare reform measures will impact our business.

Other legislative changes have been proposed and adopted in the United States since the Affordable Care Act was enacted. For example, the U.S. American Rescue Plan Act of 2021 eliminated the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, starting January 1, 2024. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation. Further, the U.S. Budget Control Act of 2011, included aggregate reductions of Medicare payments to providers of 2% per fiscal year that will remain in effect through 2031. The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws and regulations may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

The Inflation Reduction Act of 2022, or the IRA, includes several provisions that will impact our business to varying degrees, depending on how various aspects of the IRA are implemented. Provisions that may impact our business include a $2,000 out-of-pocket cap for Medicare Part D beneficiaries, the imposition of new manufacturer financial liability on most drugs in Medicare Part D, permitting the U.S. government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition, requiring companies to pay rebates to Medicare for drug prices that increase faster than inflation, and delay until January 1, 2032 the implementation of the HHS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The effect of IRA on our business and the healthcare industry in general is not yet known.

Other U.S. Environmental, Health and Safety Laws and Regulations

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, Our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintains workers' compensation insurance to cover costs and expenses we may incur due to injuries to our employees as well as insurance for environmental liability, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Employees and Human Capital Resources

As of December 31, 2024, we had 84 full-time employees, including 34 who hold Ph.D. or M.D. degrees, and no part-time employees. Of the full-time employees, 60 employees are engaged in research and development and 24 employees are engaged in

management or selling, general and administrative activities. None of our employees are subject to a collective bargaining agreement or represented by a trade or labor union. We consider our relationship with our employees to be good.

Our human capital objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

Facilities

Our principal office is located at 321 Arsenal Street, Suite 101, Watertown, MA 02472, where we lease and sublease a total of approximately 16,847 square feet of office space. The lease and sublease terms will end in December 2029 and November 2026, respectively. We believe that these facilities will be adequate for our near-term needs. If required, we believe that suitable additional or substitute space will be available in the future on commercially reasonable terms to accommodate any such expansion of our operations.

Legal Proceedings

From time to time, we may be involved in various other claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings.

Available Information

Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, proxy statements, and other information, including amendments and exhibits to such reports, filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, are available free of charge on our website located at www.discmedicine.com, as soon as reasonably practicable after they are filed with or furnished to the SEC. These reports are also available at the SEC's Internet website at www.sec.gov.

ITEM 1A. RISK FACTORS

Set forth below are the risks that we believe are material to our investors and they should be carefully considered. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and other factors not presently known to us or that we currently believe are immaterial may affect our business, prospects, financial condition and results of operations if they occur. This section contains forward-looking statements. You should refer to the explanation of the qualifications and limitations on forward-looking statements beginning on page ii of this Annual Report on Form 10-K.

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2017 and are a clinical-stage biopharmaceutical company with a limited operating history. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. Since our inception in October 2017, we have devoted substantially all of our efforts to organizing and staffing our company, business planning, capital raising, establishing and maintaining our intellectual property portfolio, building our pipeline of product candidates, conducting drug discovery activities, undertaking preclinical studies, conducting early-stage clinical trials, preparing for the potential commercialization of bitopertin, if approved, and providing general and administrative support for these operations. We have not yet demonstrated our ability to successfully develop any product candidate, obtain regulatory approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

In addition, as our business grows, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We have incurred significant net losses since our inception and anticipate that we will continue to incur losses for the foreseeable future.

Our net losses were $109.4 million and $76.4 million for the years ended December 31, 2024 and 2023, respectively. We had an accumulated deficit of $298.0 million as of December 31, 2024. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from selling, general and administrative costs associated with our operations. We expect our research and development expenses to increase significantly in connection with the commencement and continuation of clinical trials of our product candidates. In addition, if we obtain regulatory approval for our product candidates, we will incur significant sales, marketing and manufacturing expenses. We also will continue to incur additional costs associated with operating as a public company and expect to continue to incur significant and increasing operating losses over the next several years and for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

The amount of our future losses is uncertain and our quarterly and annual operating results may fluctuate significantly in the future due to a variety of factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing and success or failure of preclinical studies and clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- our ability to successfully open clinical trial sites and recruit and retain subjects for clinical trials and any delays caused by difficulties in such efforts;
- our ability to obtain regulatory approval for our product candidates, and the timing and scope of any such approvals we may receive;
- the timing and cost of, and level of investment in, research and development activities relating to our product candidates, which may change from time to time;
- the cost of manufacturing our product candidates and products, should they receive regulatory approval, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- our ability to attract, hire and retain qualified personnel;

- expenditures that we will or may incur to develop additional product candidates;
- the level of demand for our products should they receive regulatory approval, which may vary significantly;
- the risk/benefit profile, cost and reimbursement policies with respect to our product candidates, if approved, and existing and potential future therapeutics that compete with our product candidates;
- the changing and volatile U.S. and global economic environments, including as a result of public health crises; and
- future accounting pronouncements or changes in our accounting policies.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. This variability and unpredictability could also result in us failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even if we have met any previously publicly stated guidance we may provide.

We have no products approved for commercial sale and have not generated any revenue from product sales.

Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated collaborative revenue from our product candidates and have not generated revenue from product sales, and do not expect to generate any revenue from the sale of products in the near future. We do not expect to generate significant revenue unless and until we obtain regulatory approval of, and begin to sell, one or more of our product candidates. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- successfully complete our ongoing and planned preclinical studies for our current and future product candidates;
- timely file and receive acceptance of our Investigational New Drug applications, or INDs, in order to commence our planned clinical trials or future clinical trials;
- successfully enroll subjects in and complete, our ongoing and planned clinical trials;
- initiate and successfully complete all safety and efficacy studies necessary to obtain U.S. and foreign regulatory approval for our product candidates;
- successfully address the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates, if any;
- timely file New Drug Applications, or NDAs, and Biologics License Applications, or BLAs, and receive regulatory approvals for our product candidates from the U.S. Food and Drug Administration, or the FDA, and comparable foreign regulatory authorities;
- establish and maintain clinical and commercial manufacturing capabilities or make arrangements with third-party manufacturers for clinical supply and commercial manufacturing;
- obtain and maintain patent and trade secret protection or regulatory exclusivity for our product candidates;
- launch commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- obtain and maintain acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- position our product candidates to effectively compete with other therapies;
- obtain and maintain healthcare coverage and adequate reimbursement;
- enforce and defend intellectual property rights and claims; and
- maintain a continued acceptable safety profile of our products following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Our existing and any future indebtedness could adversely affect our ability to operate our business.

In November 2024, we entered into a Loan and Security Agreement, or the Hercules Loan Agreement, with the lenders party thereto, or the Lenders, and Hercules Capital, Inc., as administrative agent and collateral agent, or the Agent. The Hercules Loan Agreement provides for up to $200.0 million of senior secured term loans, or the Term Loan, available to us in multiple tranches. The Term Loan will mature on December 1, 2029. Our obligations under the Hercules Loan Agreement are secured, subject to customary permitted liens and other agreed-upon exceptions, by a first-priority perfected security interest in all of our tangible and intangible assets, other than intellectual property.

The Hercules Loan Agreement contains customary affirmative and negative covenants that could prevent us from taking certain actions without the consent of the Lenders. These covenants may limit our flexibility in operating our business and our ability to take actions that might be advantageous to us and our stockholders. Affirmative covenants include, among others, a financial covenant requiring us to maintain a minimum amount of qualified cash. Negative covenants include, among others: restrictions on transferring any part of our business or intellectual property; incurring additional indebtedness; engaging in mergers or acquisitions; paying dividends or making other distributions; making investments; and creating other liens on our assets, in each case subject to customary exceptions.

The Hercules Loan Agreement also contains customary events of default, including the occurrence of a Material Adverse Effect (as defined in the Hercules Loan Agreement), and failure to make payments when due or comply with other covenants therein. We intend to satisfy our current and future debt service obligations with our then-existing cash and cash equivalents. However, we may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under Hercules Loan Agreement or any other debt instruments. Upon the occurrence and continuance of an event of default, the Lenders may accelerate all of our repayment obligations and take control of our pledged assets, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. Any declaration by the Lenders of an event of default would therefore significantly harm our business and prospects and could cause the price of our common stock to decline.

If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

We may need to raise substantial additional funding. If we are unable to raise capital when needed or on terms acceptable to us, we may be forced to delay, reduce or eliminate some of our product development programs or commercialization efforts.

The development of pharmaceutical products is capital-intensive. We are currently advancing our hematologic disease programs through preclinical and clinical development. We expect our expenses to significantly increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate and complete clinical trials of, and seek regulatory approval for, our product candidates. In addition, depending on the status of regulatory approval or, if we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. We may also need to raise additional funds sooner if we choose to pursue additional indications and/or geographies for our current or future product candidates or otherwise expand more rapidly than presently anticipated. Furthermore, we expect to continue to incur costs associated with operating as a public company. Accordingly, we may need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate certain of our research and development programs or future commercialization efforts.

We believe that our cash, cash equivalents and marketable securities, including the net proceeds of our January 2025 underwritten public offering, will enable us to fund operating expenses and capital expenditure requirements into 2028, without taking into account any potential net cash inflows from bitopertin or any other marketed product, if approved during such period. However, we have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than expected. Our future capital requirements will depend on and could increase significantly as a result of many factors, including:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to raise additional funds to the extent necessary to complete clinical development of and commercialize our product candidates;
- our ability to establish new and maintain existing licensing or collaboration arrangements and the progress of the development efforts of third parties with whom we may enter into such arrangements;
- our ability to maintain our current research and development programs and to establish new programs;
- the successful initiation, enrollment and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;

- the receipt and related terms of regulatory approvals from applicable regulatory authorities for any product candidates;
- the availability of raw materials for use in production of our product candidates;
- establishing agreements with third-party manufacturers for supply of product candidate components for our clinical trials;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our other rights in our intellectual property portfolio;
- commercializing product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement for any approved products; and
- the potential additional expenses attributable to adjusting our development plans (including any supply related matters) to any public health crises.

Identifying potential product candidates and conducting preclinical development testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will be derived from sales of products that may take longer than we anticipate to become commercially available, if they become commercially available at all. Accordingly, we may need to continue to rely on additional financing to achieve our business objectives.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Disruptions in the financial markets in general may make equity and debt financing more difficult to obtain and may have a material adverse effect on our ability to meet our fundraising needs. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product that has received regulatory approval or be unable to expand our operations or otherwise capitalize on our business opportunities as desired, which could materially affect our business, financial condition and results of operations.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, we can generate substantial product revenue, we expect to finance our cash needs through a combination of private and public equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. Other than up to $80.0 million of additional advances that may be drawn at our option under the Hercules Loan Agreement, we do not have any committed external source of funds. The terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest will be diluted, and the terms of those securities may include liquidation or other preferences that may materially adversely affect your rights as a common stockholder. Additional debt financing, if available, would increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, acquiring, selling or licensing intellectual property rights, and making capital expenditures, declaring dividends or other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to meet certain milestones in connection with debt financing and the failure to achieve such milestones by certain dates may force us to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us which could have a material adverse effect on our business, operating results and prospects.

We also could be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable. If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or product candidates, grant licenses on terms that may not be favorable to us or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourself, any of which may have a material adverse effect on our business, operating results and prospects.

Risks Related to the Discovery and Development of Our Product Candidates

We have not yet progressed any product candidates into a Phase 3 clinical trial and may be unable to successfully complete any additional clinical trials for any product candidates we develop. Certain of our programs are still in preclinical development and may never advance to clinical development.

We currently have only three product candidates in clinical development, none of which has yet progressed into a Phase 3 clinical trial. As such, we have not yet demonstrated our ability to successfully complete large-scale, pivotal clinical trials, obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful commercialization. The majority of our programs are still in preclinical and early to mid-stage clinical development. Our clinical programs may not advance to the next stage of clinical development, and our preclinical programs may never advance to clinical development or through clinical development, as applicable. We may not initiate any clinical trial of our product candidates until we have submitted an IND to the FDA or comparable submissions with equivalent regulatory authorities and received regulatory clearance. We may not be able to submit INDs or other regulatory filings for bitopertin or any of our other product candidates on the timelines we expect, if at all. For example, we may experience manufacturing delays or other delays with IND-enabling studies. Moreover, we cannot be sure that submission of regulatory filings with the FDA or other regulatory authorities will result in such regulatory authorities allowing clinical trials to begin on a timely basis or at all, or that, once begun, such trials will be completed on schedule, if at all, or that issues will not arise that require us to revise, postpone, suspend or terminate our clinical trials. For example, we filed an IND in April 2022 with the FDA to initiate the AURORA Phase 2 trial of bitopertin in EPP patients, but the FDA initially placed the initiation of this trial on clinical hold; we received clearance to initiate the study in July 2022 after the study design was finalized with the FDA, and we initiated the study in October 2022. Commencing any of our clinical trials is subject to finalizing the trial design based on discussions with the FDA and other regulatory authorities. Any guidance we receive from the FDA or other regulatory authorities is subject to change. These regulatory authorities could change their position, including on the acceptability of our trial designs or the clinical endpoints selected, which may require us to complete additional clinical trials or result in the composition of stricter approval conditions than currently expected. For a further example, we relied on the data package generated by F. Hoffmann-La Roche Ltd. and Hoffmann-La Roche Inc., or collectively, Roche, to support our IND submission for bitopertin to initiate our AURORA Phase 2 clinical trial in patients with EPP, as well as our submission of an application with the Australian Therapeutic Goods Administration, or TGA, for our BEACON Phase 2 clinical trial in patients with EPP or XLP, and it is possible that the FDA or TGA, as applicable, may require us to conduct additional preclinical studies to support a future marketing application of bitopertin. Successful completion of our clinical trials is a prerequisite to submitting an NDA or a BLA to the FDA, a Marketing Authorisation Application, or MAA, to the European Medicines Agency, or EMA, or other marketing applications to regulatory authorities in other jurisdictions, for each product candidate and, consequently, the regulatory approval of each product candidate.

A single well-controlled clinical trial may not be sufficient for approval. In general, the FDA requires two well-controlled clinical trials to support registration of a new drug or biologic. Exceptions may be made in cases of a severe disease with few treatment options, and in principle this exception may appear applicable to many of the diseases that we seek to treat, such as EPP, XLP, anemia of MF, DBA and others. Nonetheless, the FDA and other regulators may always require additional clinical trials to support regulatory approval.

If we are required to conduct additional preclinical studies or clinical trials or other testing of our product candidates beyond those that are currently contemplated, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining regulatory approval for our product candidates;
- not obtain regulatory approval at all;
- obtain regulatory approval for indications or patient populations that are not as broad as intended or desired;
- continue to be subject to post-marketing testing requirements; or
- experience having the product removed from the market after obtaining regulatory approval.

Our programs are focused on the development of therapeutics for patients with hematologic diseases, which is a rapidly evolving area of science, and the approach we are taking to discover and develop product candidates is novel and may never lead to approved or marketable products.

The discovery and development of therapeutics for patients with hematologic diseases is an emerging field, and the scientific discoveries that form the basis for our efforts to discover and develop product candidates are relatively new. The scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although we believe, based on our preclinical work and clinical results to date, that our programs have the potential to provide disease-modifying therapies, future clinical results may not confirm this hypothesis or may only confirm it for certain alterations or certain indications.

The patient populations for our product candidates are limited to those with specific hematologic diseases. We cannot be certain that the patient populations for each specific disease will be large enough to allow us to successfully obtain approval for and commercialize our product candidates and achieve profitability.

Clinical product development involves a lengthy and expensive process, with an uncertain outcome.

Our preclinical studies and future and ongoing clinical trials may not be successful. Currently, the majority of our programs are in preclinical and early to mid-stage clinical development. It is impossible to know when or if any of our product candidates will prove effective and safe in humans or will receive regulatory approval. Before obtaining regulatory approval from regulatory authorities for the sale of any product candidate, we must complete preclinical studies and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates or the safety, purity and potency of our biological product candidates in humans. There is no guarantee that our product candidates will advance in accordance with the timelines we anticipate, if at all. Clinical testing is expensive, difficult to design and implement, can take many years to complete and outcomes are uncertain. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical development testing and early clinical trials may not be predictive of the success of later clinical trials, and interim, top-line, initial or preliminary results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain regulatory approval of their product candidates. Our preclinical studies and future and ongoing clinical trials may not be successful.

Additionally, some of the clinical trials we conduct, such as our completed BEACON Phase 2 clinical trial of bitopertin and our ongoing Phase 2 clinical trial of DISC-0974, may be open-label in study design and may be conducted at a limited number of clinical sites on a limited number of patients. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label clinical trial may not be predictive of future clinical trial results when studied in a controlled environment with a placebo or active control.

In May 2021, we entered into a license agreement with Roche, or the Roche Agreement, pursuant to which, among other things, Roche granted us an exclusive and sublicensable (subject to Roche's consent, except with respect to affiliates) worldwide license under certain of Roche's patent rights and know-how to develop and commercialize bitopertin. Although bitopertin was originally evaluated by Roche in over 4,000 individuals, Roche did not evaluate bitopertin in EPP or XLP, so the safety data generated from Roche's clinical trials of bitopertin may not be predictive or indicative of the results of our clinical trials. Regulatory authorities may also raise questions regarding the transition in the future from Roche-manufactured drug substance to drug substance manufactured by us or another party, and we may be required to conduct comparability assessments, which could result in delays in development and additional costs. We may face similar challenges with respect to our other product candidates, for which preclinical results may not be indicative or predictive of future clinical trial results.

Because we are developing some of our product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to predict or provide clinically meaningful results.

Many of our product candidates are designed to treat diseases for which there are few available therapeutic options. For example, in the United States there are currently no therapies approved to treat anemia of MF and there is only one approved therapy to treat EPP. As a result, the design and conduct of clinical trials of product candidates for the treatment of these diseases may take longer, be more costly or be less effective as part of the novelty of development in these diseases. In some cases, we may use new or novel endpoints or methodologies. The FDA or other regulatory authorities may not consider the endpoints of our clinical trials to be validated or clinically meaningful and we may need to conduct proof-of-concept studies or additional work to refine our endpoints and inform the design of future studies before initiating pivotal studies of our product candidates. Even if applicable regulatory authorities do not object to our proposed endpoints in an earlier stage clinical trial, such regulatory authorities may require evaluation of additional or different clinical endpoints in later-stage clinical trials.

Even if the FDA does find our clinical trial success criteria to be sufficiently supported and clinically meaningful at the time, we may not achieve the pre-specified endpoint to a degree of statistical significance in any pivotal or other clinical trials we may conduct for our product candidates. Further, even if we do achieve the pre-specified criteria, our trials may produce results that are unpredictable or inconsistent with the results of the more traditional efficacy endpoints in the trial. The FDA also could change its

view or give overriding weight to other efficacy endpoints over a primary endpoint, even if we achieve statistically significant results on that primary endpoint, if for example we do not do so on our secondary efficacy endpoints. The FDA also weighs the benefits of a product candidate against its risks and the FDA may view the efficacy results in the context of safety as not being supportive of approval. Other regulatory authorities in Europe and other countries may make similar findings with respect to these endpoints.

Interim, top-line, initial and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to confirmation, audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, top-line, initial or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data. For example, we announced initial data from our Phase 1b/2 DISC-0974 trial in patients with anemia of MF in December 2023, June 2024, and December 2024, and from our Phase 1b DISC-0974 trial in patients with anemia and NDD-CKD in December 2023 and October 2024. We also announced initial data from the SAD portion of the Phase 1 clinical trial of DISC-3405 in healthy volunteers in June 2024. We also may make assumptions, estimations, calculations and conclusions as part of our analyses of data, and may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, top-line, initial or preliminary results that we report may differ from future results of the same trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Interim, top-line, initial and preliminary data from clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Interim, top-line, initial and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the interim, top-line, initial or preliminary data we previously published. As a result, interim, top-line, initial and preliminary data should be viewed with caution until the final data are available. Adverse differences between interim, top-line, initial or preliminary data and final data could significantly harm our business prospects and may cause the price of our common stock to fluctuate or decline.

Further, regulatory agencies and others, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could adversely impact the potential of the particular program, the likelihood of obtaining regulatory approval of the particular product candidate, commercialization of any approved product and the business prospects of the company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is derived from information that is typically extensive, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure.

If the interim, top-line, initial or preliminary data that we report differs from actual results, or if regulatory authorities or others, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be significantly impaired, which could materially harm our business, operating results, prospects or financial condition.

We may incur additional costs or experience delays in initiating or completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

We may experience delays in initiating or completing our preclinical studies or clinical trials, including as a result of delays in obtaining, or failure to obtain, the FDA's authorization to initiate clinical trials under future INDs. Additionally, we cannot be certain that preclinical studies or clinical trials for our product candidates will not require redesign, will enroll an adequate number of subjects on time, or will be completed on schedule, if at all. We may experience numerous unforeseen events during, or as a result of, preclinical studies and clinical trials that could delay or prevent our ability to receive regulatory authorizations, regulatory approval or commercialize our product candidates, including:

- we may receive feedback from regulatory authorities that requires us to modify the design or implementation of our preclinical studies or clinical trials or to delay or terminate a clinical trial;
- regulators or institutional review boards, or IRBs, or ethics committees may delay or may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective clinical research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- preclinical studies or clinical trials of our product candidates may fail to show safety or efficacy or otherwise produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials, or we may decide to abandon product research or development programs;

- preclinical studies or clinical trials of our product candidates may not produce differentiated or clinically significant results across indications;
- the number of patients required for clinical trials of our product candidates may be larger than anticipated, enrollment in these clinical trials may be slower than anticipated or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than anticipated;
- our third-party contractors may fail to comply with regulatory requirements, fail to maintain adequate quality controls, be unable to provide us with sufficient product supply to conduct or complete preclinical studies or clinical trials, fail to meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators or IRBs or ethics committees may require us or our investigators to, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants in our clinical trials are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than anticipated;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate, and any transfer of manufacturing activities may require unforeseen manufacturing or formulation changes;
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us, regulators or IRBs or ethics committees to suspend or terminate the trials, or reports may arise from preclinical or clinical testing of other hematologic disease therapies that raise safety or efficacy concerns about our product candidates;
- any future collaborators may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us; and
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as anticipated.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs or ethics committees of the institutions at which such trials are being conducted or by the FDA or other regulatory authorities, or if the Data Safety Monitoring Board, or DSMB, for such trial recommends suspension or termination of the trial. Such authorities may impose or recommend such a suspension or termination or clinical hold due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, adverse findings upon an inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. For example, we filed an IND in April 2022 with the FDA to initiate the AURORA Phase 2 trial of bitopertin in EPP patients, but the FDA initially placed the initiation of this trial on clinical hold; we received clearance to initiate the study in July 2022 after the study design was finalized with the FDA and initiated the study in October 2022. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. Further, the FDA may disagree with our clinical trial design or our interpretation of data from clinical trials or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials.

Moreover, principal investigators for our current and future clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected the interpretation of the trial. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site, and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of regulatory approval of one or more of our product candidates.

Our product development costs will also increase if we experience delays in testing or regulatory approvals. We do not know whether any of our future clinical trials will begin as planned, or whether any of our current or future clinical trials will need to be restructured or will be completed on schedule, if at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which would impair our ability to successfully commercialize our product candidates and may significantly harm our business, operating results, financial condition and prospects.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or comparable foreign regulatory authorities, or as needed to provide appropriate statistical power for a given trial. In particular, because we are focused on patients with specific rare hematologic diseases for the development of our product candidates, our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate.

We may experience difficulties with identifying specific patient populations for any defined trial cohorts. The patient eligibility criteria defined in our trial protocols may limit the patient populations eligible for our clinical trials. We will also rely on the willingness and ability of clinicians to screen their patients, such as for specific genetic hematologic conditions, to indicate which patients may be eligible for enrollment in our clinical trials.

In addition, some of our competitors have ongoing clinical trials for product candidates that are intended to treat the same indications as our product candidates, and patients who would otherwise be eligible for our clinical trials may choose instead to enroll in clinical trials of our competitors' product candidates.

Additionally, the process of finding patients may prove costly. We also may not be able to identify, recruit or enroll a sufficient number of patients to complete our clinical trials because of the small patient populations with rare hematologic diseases, the perceived risks and benefits of the product candidates under study, the availability and efficacy of competing therapies and clinical trials, the proximity and availability of clinical trial sites for prospective patients, and the patient referral practices of physicians. If patients are unwilling to participate in our studies for any reason, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of potential products may be delayed.

Patient enrollment may be affected by other factors, including:

- the severity of the disease under investigation;
- the efforts to obtain and maintain patient consents and facilitate timely enrollment in clinical trials;
- the ability to monitor patients adequately during and after treatment;
- the risk that patients enrolled in clinical trials will drop out of the clinical trials before clinical trial completion;
- the ability to recruit clinical trial investigators with the appropriate competencies and experience;
- reporting of the preliminary results of any of our clinical trials; and
- factors we may not be able to control, including the impacts of any public health crises, that may limit patients, principal investigators or staff or clinical site availability.

Results from early preclinical studies and clinical trials of our programs and product candidates are not necessarily predictive of the results of later preclinical studies and clinical trials of our programs and product candidates. If we cannot replicate the results from earlier preclinical studies and clinical trials of our programs and product candidates in our later preclinical studies and clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.

Any results from early preclinical studies and clinical trials of bitopertin, DISC-0974, DISC-0998, DISC-3405 or our other product candidates or programs may not necessarily be predictive of the results from later preclinical studies and clinical trials. For example, we have announced positive initial data from a Phase 1b/2 clinical trial of DISC-0974 in patients with non-dialysis dependent CKD and anemia. However, there can be no assurance that DISC-0974 will achieve the desired effects in this indication. Additionally, we announced positive results from a Phase 1 clinical trial of DISC-3405 in healthy adult volunteers in December 2024, which may not be indicative or predictive of future clinical trial results. Similarly, even if we are able to complete our planned preclinical studies and clinical trials of our product candidates according to our current development timeline, the results from such preclinical studies and clinical trials of our product candidates may not be replicated in subsequent preclinical studies or clinical trial results.

Many companies in the biopharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway, or safety or efficacy observations made in preclinical studies and clinical trials, including previously unreported adverse events. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain regulatory approval.

Our clinical trials or those of our future collaborators may reveal significant adverse events not seen in prior preclinical studies or clinical trials and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

Before obtaining regulatory approvals for the commercial sale of any products, we must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that our product candidates regulated as drugs are safe and effective and our product candidates regulated as biologics are safe, pure and potent for use in each target indication. Clinical testing is expensive and can take many years to complete, and outcomes are inherently uncertain. Failure can occur at any time during the clinical trial process. Because the majority of our programs and product candidates are in an early stage of development, there is a high risk of failure, and we may never succeed in developing marketable products. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials also may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. For example, Roche had previously developed bitopertin as a potential therapy for certain symptoms of schizophrenia and obsessive-compulsive disorder, but discontinued the program for lack of efficacy in those indications after completing over 30 clinical trials in over 4,000 individuals. If the results of our ongoing or future preclinical studies and clinical trials are inconclusive with respect to the safety and efficacy of our programs and product candidates, if we do not meet the clinical endpoints with statistical and clinically meaningful significance, or if there are safety concerns associated with our product candidates, we may be prevented from, or delayed in, obtaining regulatory approval for such product candidates. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols and the rate of dropout among clinical trial participants. Results of our trials could reveal a high and unacceptable severity and prevalence of side effects. In such an event, our trials could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

Further, our product candidates could cause undesirable side effects in clinical trials related to on-target toxicity. If on-target toxicity is observed, or if our product candidates have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. In addition, our product candidates could cause undesirable side effects that have not yet been observed. For example, bitopertin may demonstrate toxicities in patients with hematologic diseases not previously observed by Roche when it was studied in different indications. Many compounds that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the compound. Most product candidates that commence clinical trials are never approved as products, and there can be no assurance that any of our current or future clinical trials will ultimately be successful or support further clinical development or regulatory approval of any of our product candidates.

As is the case with many treatments for hematologic and rare diseases, it is likely that there may be side effects associated with the use of our product candidates. If significant adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to our clinical trials, patients may drop out of our trials, or we may be required to abandon the trials or development efforts of one or more product candidates altogether. We, the FDA or other applicable regulatory authorities, or an IRB may suspend or terminate clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Even if the side effects do not preclude the product from obtaining or maintaining regulatory approval, undesirable side effects may inhibit market acceptance of the approved product due to its tolerability versus other therapies. Any of these developments could materially harm our business, operating results, financial condition and prospects.

Some of our product candidates modulate pathways for which there are currently no approved or effective therapies, which may result in greater research and development expenses, regulatory issues that could delay or prevent approval or discovery of unknown or unanticipated adverse effects on safety or efficacy.

Some of our product candidates modulate pathways for which there are currently no approved or effective therapies, which may result in uncertainty. We select programs for targets based on compelling biological rationale, including evidence of expected biological effects in humans. We explore new programs based on extensive preclinical data analysis which sometimes cannot predict efficacy or safety in humans. Regulatory approval of novel product candidates such as ours can be more expensive, riskier and take longer than for other, more well-known or extensively studied pharmaceutical or biopharmaceutical product candidates due to our and regulatory agencies' lack of experience with them. The novelty of the mechanism of action of any of our product candidates may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. The novel mechanism of action also means that fewer people are trained in or experienced with product candidates of this type, which may make it more difficult to find, hire and retain

personnel for research, development and manufacturing positions. If our product candidates utilize a novel mechanism of action that has not been the subject of extensive study compared to more well-known product candidates, there is also an increased risk that we may discover previously unknown or unanticipated adverse effects during our preclinical studies and clinical trials. Our product candidates may achieve lower efficacy in patients than expected. Any such events could adversely impact our business prospects, operating results and financial condition.

We have conducted and are currently conducting clinical trials for bitopertin in Australia and may in the future conduct additional clinical trials of our product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.

We conducted BEACON, our Phase 2 open-label, parallel-dose clinical trial of bitopertin in EPP and XLP patients, at sites in Australia. All participants in BEACON are eligible to participate in HELIOS, an open-label, long-term extension study of bitopertin in EPP and XLP patients, that we are also conducting in Australia. We are also planning to conduct APOLLO, a randomized, double-blind, placebo-controlled clinical trial of bitopertin in EPP and XLP patients, in Canada, Europe and Australia, as well as the United States. We may in the future choose to conduct additional clinical trials of our product candidates outside the United States, including in Europe, Australia, or other foreign jurisdictions. The acceptance of trial data from clinical trials conducted outside the United States by the FDA may be subject to certain conditions. In cases where data from clinical trials conducted outside the United States are intended to serve as the sole basis for regulatory approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practices, (ii) the trials were performed by clinical investigators of recognized competence and (iii) the data may be considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory bodies have similar approval requirements. In addition, such foreign trials will be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority, will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving regulatory approval or clearance for commercialization in the applicable jurisdiction.

Although we intend to explore other therapeutic opportunities in addition to the programs and product candidates that we are currently developing, we may fail to identify viable new product candidates for clinical development for a number of reasons. If we fail to identify additional product candidates, our business could be materially harmed.

Research programs to pursue the development of our existing and planned product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not they are ultimately successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective products; or
- it may take greater human and financial resources than we will possess to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, thereby limiting our ability to develop, diversify and expand our product portfolio.

Because we have limited financial and human resources, we intend to initially focus on research programs and product candidates for a limited set of indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or to develop suitable product candidates through internal research programs, which could materially adversely affect our future growth and prospects. We may focus our efforts and resources on potential product candidates or other potential programs that ultimately prove to be unsuccessful.

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for any of our product candidates, we will not be able to commercialize, or will be delayed in commercializing, such product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable foreign regulatory authorities. Before we can commercialize any of our product candidates, we must obtain regulatory approval. Currently, all of our product candidates are in discovery, preclinical or clinical development, and we have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. It is possible that our product candidates, including any product candidates we may seek to develop in the future, will never obtain regulatory approval. We have limited experience in filing and supporting the applications necessary to gain regulatory approvals and rely on third-party CROs and/or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude us from obtaining regulatory approval or prevent or limit commercial use. In addition, regulatory authorities may find fault with our manufacturing process or facilities or that of third-party contract manufacturers. We may also face greater than expected difficulty in manufacturing our product candidates.

The process of obtaining regulatory approvals, both in the United States and abroad, is expensive and often takes many years. If the FDA or a comparable foreign regulatory authority requires that we perform additional preclinical studies or clinical trials, approval may be delayed, if obtained at all. The length of such a delay varies substantially based upon a variety of factors, including the type, complexity and novelty of the product candidate involved. Changes in regulatory approval policies during the development period, changes in or enactment of additional statutes or regulations, or changes in regulatory review policies for each submitted NDA, BLA, or equivalent application types, may cause delays in the approval or rejection of an application. The FDA and comparable foreign regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. Our product candidates could be delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may not be able to enroll a sufficient number of patients in our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA, BLA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change such that our clinical data are insufficient for approval.

Even if we were to obtain regulatory approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, thereby narrowing the commercial potential of the product candidate. In addition, regulatory authorities may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

If we experience delays in obtaining, or if we fail to obtain, approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenue will be materially impaired.

A public health crisis, pandemic, epidemic, or outbreak of an infectious or highly contagious disease, may materially and adversely affect our business and financial results and could cause a disruption to the development of our product candidates.

Public health crises such as pandemics or similar outbreaks could adversely impact our business. The extent to which an outbreak of highly infectious or contagious diseases impacts our operations or those of our third-party partners, including our preclinical studies or clinical trial operations, will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the scope, severity and duration of the outbreak, actions taken to contain the outbreak or mitigate its impact, and the direct and indirect economic effects of the outbreak and containment measures, among others.

In addition, the patient populations that our product candidates target may be particularly susceptible to highly infectious or contagious diseases, which may make it more difficult for us to identify patients able to enroll in our current and future clinical trials and may impact the ability of enrolled patients to complete any such trials.

Risks Related to Commercialization

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new products in the biopharmaceutical and related industries is highly competitive. We compete in the segments of the pharmaceutical, biotechnology, and other related markets that develop therapies in the field of hematologic diseases. There are other companies focusing on developing therapies in the field of hematologic diseases. We also compete more broadly across the market for cost-effective and reimbursable treatments. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. These companies include divisions of large pharmaceutical companies and biotechnology companies of various sizes. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. See “Business - Competition” for examples of the competition that our product candidates face.

Any product candidates that we successfully develop and commercialize will compete with currently approved therapies and new therapies that may become available in the future from segments of the pharmaceutical, biotechnology and other related markets. Key product features that would affect our ability to effectively compete with other therapeutics include the efficacy, safety and convenience of our products. We believe principal competitive factors to our business include, among other things, our ability to successfully transition research programs into clinical development, ability to raise capital and the scalability of the platform, pipeline and business.

Many of the companies that we compete against or which we may compete against in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical and clinical testing, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. If these or other barriers to entry do not remain in place, other companies may be able to more directly or effectively compete with us.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we or our collaborators may develop. Our competitors also may obtain FDA or other regulatory approval for their products sooner than we may obtain approval for our product candidates, which could result in our competitors establishing a strong market position before we or our collaborators are able to enter the market. The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, level of generic competition and availability of reimbursement from government and other third-party payors.

If the market opportunities for our programs and product candidates are smaller than we estimate or if any regulatory approval that we obtain is based on a narrower definition of the patient population, our revenue and ability to achieve profitability could be materially adversely affected.

The incidence and prevalence for the target patient populations of our programs and product candidates have not been established with precision. Our lead heme biosynthesis modulation product candidate, bitopertin, is an oral, selective inhibitor of GlyT1. We are initially focused on developing bitopertin for the treatment of EPP and XLP, which are both diseases marked by severe photosensitivity and damage to the hepatobiliary system caused by the accumulation of PPIX. We are initially focused on developing DISC-0974 for anemia of MF and anemia of non-dialysis dependent CKD. We are initially focused on developing DISC-3405 for the treatment of PV. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our programs and product candidates, are based on our estimates.

The total addressable market opportunity will ultimately depend upon, among other things, the diagnosis criteria included in the final label, the indications for which our product candidates are approved for sale, acceptance by the medical community and patient access, product pricing and reimbursement. The number of patients with erythropoietic porphyria and anemias of inflammation for which our product candidates may be approved as treatment may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. We may not be successful in our efforts to identify additional product candidates. Due to our limited resources and access to capital, we must prioritize development of certain product candidates, which may prove to be the wrong choice and may adversely affect our business.

If our current product candidates or any future product candidates do not achieve broad market acceptance, the revenue that we generate from our sales may be limited, and we may never become profitable.

We have never commercialized a product candidate for any indication. Even if our current product candidates and any future product candidates are approved by the appropriate regulatory authorities for marketing and sale, they may not gain acceptance among physicians, patients, third-party payors, and others in the medical community. If any product candidates for which we may obtain regulatory approval do not gain an adequate level of market acceptance, we may not generate significant revenue and may not become profitable or may be significantly delayed in achieving profitability. Market acceptance of our current product candidates and any future product candidates by the medical community, patients and third-party payors will depend on a number of factors, some of which are beyond our control. For example, physicians are often reluctant to switch their patients, and patients may be reluctant to switch, from existing therapies even when new and potentially more effective or safer treatments enter the market. If public perception is influenced by claims that the use of heme biosynthesis modulation therapies or hepcidin-targeted agents is unsafe, whether related to our or our competitors' products, our products may not be accepted by the general public or the medical community. Future adverse events in the hematologic diseases or the biopharmaceutical industry could also result in greater governmental regulation, stricter labeling requirements and potential regulatory delays in the testing or approvals of our product candidates.

In the United States and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Our ability to successfully commercialize our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels.

Efforts to educate the medical community and third-party payors on the benefits of our current product candidates and any future product candidates may require significant resources and may not be successful. If our current product candidates or any future product candidates are approved but do not achieve an adequate level of market acceptance, we could be prevented from or significantly delayed in achieving profitability. The degree of market acceptance of any of our current product candidates and any future product candidates will depend on a number of factors, including:

- the efficacy of our current product candidates and any future product candidates;
- the prevalence and severity of adverse events associated with our current product candidates and any future product candidates;
- the clinical indications for which our product candidates are approved and the approved claims that we may make for the products;

- limitations or warnings contained in the product's FDA-approved labeling or those of comparable foreign regulatory authorities, including potential limitations or warnings for our current product candidates and any future product candidates that may be more restrictive than other competitive products;
- changes in the standard of care for the targeted indications for our current product candidates and any future product candidates, which could reduce the marketing impact of any claims that we could make following FDA approval or approval by comparable foreign regulatory authorities, if obtained;
- the relative convenience and ease of administration of our current product candidates and any future product candidates;
- the cost of treatment compared with the economic and clinical benefit of alternative treatments or therapies;
- the availability of adequate coverage or reimbursement by third-party payors, including government healthcare programs such as Medicare and Medicaid and other healthcare payors;
- the price concessions required by third-party payors to obtain coverage;
- the willingness of patients to pay out-of-pocket in the absence of adequate coverage and reimbursement;
- the extent and strength of our marketing and distribution of our current product candidates and any future product candidates;
- the safety, efficacy, and other potential advantages over, and availability of, alternative treatments already used or that may later be approved;
- distribution and use restrictions imposed by the FDA or comparable foreign regulatory authorities with respect to our current product candidates and any future product candidates or to which we agree as part of a Risk Evaluation and Mitigation Strategy, or REMS, or voluntary risk management plan;
- the timing of market introduction of our current product candidates and any future product candidates, as well as competitive products;
- our ability to offer our current product candidates and any future product candidates for sale at competitive prices;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the extent and strength of our third-party manufacturer and supplier support;
- the approval of other new products;
- adverse publicity about our current product candidates and any future product candidates or favorable publicity about competitive products; and
- potential product liability claims.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree.

We may not be successful in addressing these or other factors that might affect the market acceptance of our product candidates. Failure to achieve widespread market acceptance of our product candidates would materially harm our business, financial condition and results of operations.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to post-market study requirements, marketing and labeling restrictions and even recall or market withdrawal if unanticipated safety issues are discovered following approval. In addition, we may be subject to penalties or other enforcement action if we fail to comply with regulatory requirements.

If the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, import, export, adverse event reporting, storage, advertising, promotion, monitoring and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and listing, as well as continued compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval. Any regulatory approvals that we receive

for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing studies, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product. The FDA may also require a REMS in order to approve our product candidates, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. For certain commercial prescription drug and biological products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the United States. Additionally, under the Food and Drug Omnibus Reform Act of 2022, or FDORA, sponsors of approved drugs and biologics must provide 6 months' notice to the FDA of any changes in marketing status, such as the withdrawal of a drug, and failure to do so could result in the FDA placing the product on a list of discontinued products, which would revoke the product's ability to be marketed. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market or voluntary or mandatory product recalls;
- manufacturing delays and supply disruptions where regulatory inspections identify observations of noncompliance requiring remediation;
- revisions to the labeling, including limitation on approved uses or the addition of additional warnings, contraindications or other safety information, including boxed warnings;
- imposition of a REMS which may include distribution or use restrictions;
- requirements to conduct additional post-market clinical trials to assess the safety of the product;
- clinical trial holds;
- fines, warning letters or other regulatory enforcement action;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. In addition, the U.S. Supreme Court's July 2024 decision to overturn established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays, and/or changes. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any regulatory approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Risks Related to Our Reliance on Third Parties

We rely on third parties to conduct our clinical trials for our product candidates, as well as potential investigator-sponsored clinical trials of our product candidates. If these third parties do not successfully carry out their contractual duties, comply with regulatory requirements, or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We do not have the ability to independently conduct clinical trials. We have relied on and expect to continue to rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct or otherwise support our clinical trials for our product candidates, including our planned APOLLO clinical trial of bitopertin in EPP and XLP, as well as any other product candidates that we develop. We may also rely on academic and private non-academic institutions to conduct and sponsor clinical trials relating to our product candidates, such as the ongoing clinical trial of bitopertin in DBA, which is being conducted by NIH under a collaborative research and development agreement. We will not control the design or conduct of any investigator-sponsored trials, and it is possible that the FDA or non-U.S. regulatory authorities will not view these investigator-sponsored trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results.

Such arrangements will likely provide us certain information rights with respect to the investigator-sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator-sponsored trials. However, we would not have control over the timing and reporting of the data from investigator-sponsored trials, nor would we own the data from the investigator-sponsored trials. If we are unable to confirm or replicate the results from the investigator-sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our product candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our product candidates, or if the data proves to be inadequate compared to the first-hand knowledge we might have gained had the investigator-sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future clinical trials ourself may be adversely affected.

We rely and expect to continue to rely heavily on these parties for execution of clinical trials for our product candidates and control only certain aspects of our activities. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on CROs or other third parties will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We, our principal investigators and our CROs are required to comply with regulations, including GCPs, for conducting, monitoring, recording and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate, and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or the EEA, and comparable foreign regulatory authorities for any products in clinical development. The FDA enforces GCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we, our principal investigators or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our future clinical trials will comply with GCPs. In addition, our clinical trials must be conducted with product candidates produced under current Good Manufacturing Practice, or cGMP, regulations. Our failure or the failure of our principal investigators or CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process, significantly increase our expenditures and could also subject us to enforcement action. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Although we designed our ongoing Phase 1b/2 clinical trials of DISC-0974 and our ongoing Phase 2 clinical trial of bitopertin, are designing our planned APOLLO clinical trial of bitopertin and our planned Phase 2 clinical trial of DISC-3405, and intend to design other future clinical trials for our product candidates, these trials are or will be conducted by CROs and we expect CROs will conduct all of our future clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, are outside of our direct control. Our reliance on third parties to conduct future clinical trials also results in less direct control over the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. If the principal investigators or CROs do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates or our development program may be materially and irreversibly harmed. If we are unable to rely on clinical data collected by our principal investigators or CROs, we could be required to repeat, extend the duration of, or increase the size of any clinical trials we conduct and this could significantly delay commercialization and require significantly greater expenditures.

If any of our relationships with these third-party principal investigators or CROs terminate, we may not be able to enter into arrangements with alternative CROs. If principal investigators or CROs do not successfully carry out their contractual obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised

due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any clinical trials such principal investigators or CROs are associated with may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize, our product candidates. As a result, we believe that our financial results and the commercial prospects for our product candidates in the subject indication would be harmed, our costs could increase and our ability to generate revenue could be delayed.

We might not realize the anticipated benefits of our current collaborations with Mabwell or NIH, or any other collaborations we enter into in the future.

Research, development, commercialization and/or strategic collaborations, including those that we have with Mabwell and NIH, are subject to numerous risks, which include the following:

- collaborators may have significant control or discretion in determining the efforts and resources that they will apply to a collaboration, and might not commit sufficient efforts and resources or might misapply those efforts and resources;
- we may have limited influence or control over the approaches to research, development and/or commercialization of product candidates in the territories in which our collaboration partners lead research, development and/or commercialization;
- collaborators might not pursue research, development and/or commercialization of collaboration product candidates or might elect not to continue or renew research, development and/or commercialization programs based on preclinical studies and/or clinical trial results, changes in their strategic focus, availability of funding or other factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators might delay, provide insufficient resources to, or modify or stop research or clinical development for collaboration product candidates or require a new formulation of a product candidate for clinical testing;
- collaborators with sales, marketing and distribution rights to one or more product candidates might not commit sufficient resources to sales, marketing and distribution or might otherwise fail to successfully commercialize those product candidates;
- collaborators might not properly maintain or defend our intellectual property rights or might use our intellectual property improperly or in a way that jeopardizes our intellectual property or exposes us to potential liability;
- collaboration activities might result in the collaborator having intellectual property covering our activities or product candidates, which could limit our rights or ability to research, develop and/or commercialize our product candidates;
- collaborators might not be in compliance with laws applicable to their activities under the collaboration, which could impact the collaboration and us;
- disputes might arise between a collaborator and us that could cause a delay or termination of the collaboration or result in costly litigation that diverts management attention and resources; and
- collaborations might be terminated, which could result in a need for additional capital to pursue further research, development and/or commercialization of our product candidates.

In addition, funding provided by a collaborator might not be sufficient to advance product candidates under the collaboration. If a collaborator terminates a collaboration or a program under a collaboration, including by failing to exercise a license or other option under the collaboration, whether because we fail to meet a milestone or otherwise, any potential revenue from the collaboration would be significantly reduced or eliminated. In addition, we will likely need to either secure other funding to advance research, development and/or commercialization of the relevant product candidate or abandon that program, the development of the relevant product candidate could be significantly delayed, and our cash expenditures could increase significantly if we are to continue research, development and/or commercialization of the relevant product candidates.

Any one or more of these risks, if realized, could reduce or eliminate future revenue from product candidates under our collaborations, and could have a material adverse effect on our business, financial condition, results of operations and/or growth prospects.

We have established collaborations with Mabwell and NIH and may seek to establish additional collaborations, and, if we are not able to establish them on commercially reasonable terms, or at all, we may have to alter our development and commercialization plans.

Our product development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and

biotechnology companies, such as our collaborations with Mabwell and NIH, for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's own evaluation of a potential collaboration. Such factors a potential collaborator will use to evaluate a collaboration may include the design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

We are also restricted by Roche's right of first negotiation under our current license agreement with them and may in the future be restricted under other license or collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay our potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

In addition, any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

We contract with third parties for the manufacture of our product candidates for preclinical development and clinical testing and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We do not currently own or operate, nor do we have any plans to establish in the future, any manufacturing facilities. Although we believe we have obtained sufficient material to produce bitopertin tablets to complete our ongoing Phase 2 clinical trial, and DISC-0974 vials to complete our ongoing Phase 1b/2 clinical trials, we cannot be sure we have correctly estimated our drug product and API requirements or that such drug product or API will not expire before we want to use it. We rely, and expect to continue to rely, on third parties such as contract development and manufacturing organizations, or CDMOs, for the manufacture of our product candidates for preclinical development and clinical testing. We also expect to rely on third parties for the commercial manufacture of our products if any of our product candidates receive regulatory approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

The facilities used by our CDMOs to manufacture our product candidates must be inspected by the FDA pursuant to pre-approval inspections that will be conducted after we submit our marketing applications to the FDA. We do not control the manufacturing process of, and will be completely dependent on, our CDMOs for compliance with cGMPs in connection with the manufacture of our product candidates. If our CDMOs cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to pass regulatory inspections and/or maintain regulatory compliance. In addition, we have no control over the ability of our CDMOs to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority finds deficiencies with or does not approve these facilities for the manufacture of our product candidates or if it finds deficiencies or withdraws any such approval in

the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

If any CDMO with whom we contract fails to perform its obligations, we may be forced to enter into an agreement with a different CDMO, which we may not be able to do on reasonable terms, if at all. In such a scenario, our clinical trials supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our product candidates may be unique or proprietary to the original CDMO and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change CDMOs for any reason, we will be required to verify that the new CDMO maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new CDMO could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. In addition, changes in manufacturers often involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacturer. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

Further, our failure, or the failure of our CDMOs, to comply with applicable regulations could result in sanctions, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, if approved, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect our business and supplies of our product candidates.

We may be unable to establish any additional agreements with CDMOs or do so on acceptable terms. Reliance on CDMOs entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Our product candidates and any products that we may develop may compete with other product candidates and approved products for access to manufacturing facilities. There are a limited number of CDMOs that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or regulatory approval. If our current CDMOs cannot perform as agreed, we may be required to replace such CDMOs. In addition, there has been increased governmental focus in the U.S. on contracting with Chinese companies for the development or manufacturing of pharmaceutical products. For example, there have been Congressional legislative proposals, such as the bill titled the BIOSECURE Act that was previously considered (and not enacted) in Congress, which would have, among other things, prohibited U.S. federal funding in connection with biotechnology equipment or services produced or provided by certain named Chinese "biotechnology companies of concern" (which includes WuXi AppTech (Hong Kong) Limited and its affiliates, or WuXi) and loans and grants to, and federal contracts with any entity that uses biotechnology equipment or services from one of these entities. The legislation would also have given the federal government the authority to name additional "biotechnology companies of concern" that are engaged in research activities with the Chinese government and that pose a risk of U.S. national security. We currently rely on certain foreign or foreign-owned third-party vendors, including WuXi and its affiliates, to manufacture certain materials used in clinical trials of our product candidates or to provide services in connection with certain clinical trials or certain discovery activities. Our engagement with these foreign and foreign-owned vendors may be subject to new U.S. legislation similar to the proposed BIOSECURE Act, investigations, sanctions, trade restrictions and other foreign regulatory requirements, which could cause us to need to identify alternate service providers, increase the cost or reduce the supply of materials available to us, delay the procurement or supply of these materials, delay or impact clinical trials, or have an adverse effect on our ability to secure significant commitments from governments to purchase our potential therapies, any of which could adversely affect our financial condition and business prospects. We continue to assess the legislation as it develops to determine the effect, if any, on our contractual relationships.

Our current and anticipated future dependence upon others for the manufacture of our product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive regulatory approval on a timely and competitive basis.

The third parties upon whom we rely for the supply of the active pharmaceutical ingredients used in our product candidates are our sole sources of supply, and the loss of any of these suppliers could significantly harm our business.

The active pharmaceutical ingredients, or API, used in certain of our product candidates are supplied to us from single-source suppliers. Our ability to successfully develop our product candidates, and to ultimately supply our commercial products in quantities sufficient to meet the market demand, depends in part on our ability to obtain the API for these products in accordance with regulatory requirements and in sufficient quantities for clinical testing and commercialization. We do not currently have arrangements in place for a redundant or second-source supply of any such API in the event any of our current suppliers of such API cease their operations for any reason. We are also unable to predict how changing global economic conditions or potential global health concerns or crises will affect our third-party suppliers and manufacturers. Regional issues may in some cases accentuate these risks. For example, the pharmaceutical industry generally, and in some instances we or other third parties on which we rely, depend on China-based suppliers or service providers for certain raw materials, products and services, or other activities. Our ability or the ability of our collaborators or such other third parties to continue to engage these China-based suppliers or service providers for certain preclinical research programs and clinical development programs could be restricted due to geopolitical developments between the United States and China, including as a result of the escalation of tariffs or other trade restrictions or if the previously proposed federal legislation known as the BIOSECURE Act or a similar law were to be enacted. Any negative impact of such matters on our third-party suppliers and manufacturers may also have an adverse impact on our results of operations or financial condition.

For all of our product candidates, we intend to identify and qualify additional manufacturers to provide such API prior to submission of an NDA to the FDA and/or an MAA to the EMA. We are not certain, however, that our single-source suppliers will be able to meet our demand for their products, either because of the nature of our agreements with those suppliers, our limited experience with those suppliers or our relative importance as a customer to those suppliers. It may be difficult for us to assess our ability to timely meet our demand in the future based on past performance. While our suppliers have generally met our demand for their products on a timely basis in the past, they may subordinate our needs in the future to their other customers.

Establishing additional or replacement suppliers for the API used in our product candidates, if required, may not be accomplished quickly. If we are able to find a replacement supplier, such replacement supplier would need to be qualified and may require additional regulatory inspection or approval, which could result in further delay. While we seek to maintain adequate inventory of the API used in our product candidates, any interruption or delay in the supply of components or materials, or our inability to obtain such API from alternate sources at acceptable prices in a timely manner could impede, delay, limit or prevent our development efforts, which could harm our business, results of operations, financial condition and prospects.

The manufacture of biologics is complex and our third-party manufacturers may encounter difficulties in production. If any of our third-party manufacturers encounter such difficulties, our ability to provide supply of product candidates for clinical trials or products for patients, if approved, could be delayed or prevented.

DISC-0974, DISC-0998, and DISC-3405 are monoclonal antibodies. Manufacturing biologics, like monoclonal antibodies, especially in large quantities, is often complex and may require the use of innovative technologies to handle living cells. Each lot of an approved biologic must undergo thorough testing for identity, strength, quality, purity and potency. Manufacturing biologics requires facilities specifically designed for and validated for this purpose, and sophisticated quality assurance and quality control procedures are necessary. Slight deviations anywhere in the manufacturing process, including filling, labeling, packaging, storage and shipping and quality control and testing, may result in lot failures, product recalls or spoilage. When changes are made to the manufacturing process, we may be required to provide preclinical and clinical data showing the comparable identity, strength, quality, purity or potency of the products before and after such changes. If microbial, viral or other contaminations are discovered at the facilities of our manufacturers, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials and adversely harm our business.

In addition, there are risks associated with large scale manufacturing for clinical trials or commercial scale including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, compliance with good manufacturing practices, lot consistency and timely availability of raw materials. Even if we obtain regulatory approval for any of our current product candidates or any future product candidates, there is no assurance that our manufacturers will be able to manufacture the approved product to specifications acceptable to the FDA or other comparable foreign regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential commercial launch of the product or to meet potential future demand. If our manufacturers are unable to produce sufficient quantities for clinical trials or for commercialization, our development and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and growth prospects.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent and other intellectual property protection for our technology and product candidates, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology and drugs may be impaired, and we may not be able to compete effectively in our market.

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the U.S. and other countries for our current or future product candidates, including our current lead product candidates, bitopertin, DISC-0974 and DISC-3405, and our other current or future programs, including DISC-0998, as well as for their respective compositions, formulations, methods used to manufacture them and methods of treatment, in addition to successfully defending these patents against third-party challenges. We seek to protect our proprietary and intellectual property position by, among other methods, filing patent applications in the U.S. and abroad related to our proprietary technology, inventions, and improvements that are important to the development and implementation of our business. Our ability to stop unauthorized third parties from making, using, selling, offering to sell, or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

We have in-licensed, and may in the future in-license, a portion of our intellectual property, and, if we fail to comply with our obligations under these license arrangements, we could lose such intellectual property rights or owe damages to the licensor of such intellectual property. In particular, we have exclusively licensed intellectual property rights from Roche to develop and commercialize bitopertin, including certain back-up compounds and derivatives, for all prophylactic and therapeutic uses. The Roche license covers know-how, and certain specified Roche patent rights, including a composition of matter patent for bitopertin that expires in 2025. We also have exclusively licensed intellectual property rights from AbbVie Deutschland GmbH & Co. KG, or AbbVie, to develop and commercialize DISC-0974 and DISC-0998. The AbbVie license covers know-how, and certain specified AbbVie patent rights, including composition of matter and methods of use patents and patent applications for DISC-0974 and DISC-0998. We also have exclusively licensed intellectual property rights from Mabwell to develop and commercialize antibody products containing Mabwell's MWTX-001, MWTX-002, and MWTX-003 antibodies. The Mabwell license covers know-how and certain specified Mabwell patent rights, including composition of matter and methods of use patents and patent applications for MWTX-001, MWTX-002 and MWTX-003.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. The degree of patent protection we require to successfully commercialize our current or future product candidates may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our patents have, or that any of our pending patent applications that mature into issued patents will include, claims with a scope sufficient to protect bitopertin, DISC-0974, DISC-3405 or our other current or future product candidates. In addition, if the breadth or strength of protection provided by our patent applications or any patents we may own or in-license is threatened, we could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the U.S. For example, in jurisdictions outside the U.S., a license may not be enforceable unless all the owners of the intellectual property agree or consent to the license. Accordingly, any actual or purported co-owner of our patent rights could seek monetary or equitable relief requiring us to pay it compensation for, or refrain from, exploiting these patents due to such co-ownership. Furthermore, patents have a limited lifespan. In the U.S., and most other jurisdictions in which we have undertaken patent filings, the natural expiration of a patent is generally twenty years after it is filed, assuming all maintenance fees are paid. Various extensions may be available, on a jurisdiction-by-jurisdiction basis; however, the life of a patent, and thus the protection it affords, is limited. Additionally, our product candidates may or may not be eligible for such extensions or we may not be able to obtain such protections due to procedural or other reasons. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, patents we may own or in-license may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing drugs similar or identical to our current or future product candidates, including generic versions of such drugs.

Other parties have developed technologies that may be related or competitive to our own, and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents, with respect to either the same compounds, methods, formulations or other subject matter, in either case that we may rely upon to dominate our patent position in the market. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until at least 18 months after the earliest priority date of the patent filing, or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in patents we may own or in-license patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

In addition, the patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Further, with respect to certain pending patent applications covering our current or future product candidates, prosecution has yet to commence. Patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the relevant patent office(s) may be significantly narrowed by the time they issue, if they ever do. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Prosecution could require that claim scope narrow such that a clinical or product candidate or program is not adequately protected by the patent. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

Even if we acquire patent protection that we expect should enable us to establish and/or maintain a competitive advantage, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the U.S. and abroad. We may become involved in post-grant proceedings such as opposition, derivation, reexamination, *inter partes* review, post-grant review, invalidation, or interference proceedings challenging our patent rights or the patent rights of others from whom we may in the future obtain licenses to such rights, in the U.S. Patent and Trademark Office, or USPTO, the European Patent Office, or EPO, or in other countries. In addition, we may be subject to a third-party submission to the USPTO, the EPO, or elsewhere, that may reduce the scope or preclude the granting of claims from our pending patent applications. Competitors may allege that they invented the inventions claimed in our issued patents or patent applications prior to us, or may file patent applications before we do. Competitors may also claim that we are infringing their patents and that we therefore cannot practice our technology as claimed under our patents or patent applications. Competitors may also contest our patents by claiming to an administrative patent authority or judge that the invention was not patent-eligible, was not original, was not novel, was obvious, and/or lacked inventive step, and/or that the patent application filing failed to meet relevant requirements relating to description, basis, enablement, clarity, and/or support; in litigation, a competitor could claim that our patents, if issued, are not valid or are unenforceable for a number of reasons. If a court or administrative patent authority agrees, we would lose our protection of those challenged patents.

In addition, we may in the future be subject to claims by our former employees or consultants asserting an ownership right in our patents or patent applications, as a result of the work they performed on our behalf. Although we generally require all of our employees, consultants and advisors and any other third parties who have access to our proprietary know-how, information or technology to assign or grant similar rights to their inventions to us, we cannot be certain that we have executed such agreements with all parties who may have contributed to our intellectual property, nor can we be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy.

An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, without payment to it, or could limit the duration of the patent protection covering our technology and current or future product candidates. Such challenges may also result in our inability to manufacture or commercialize our current or future product candidates without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if they are unchallenged, our issued patents and our pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent patents we may own or in-license by developing similar or alternative technologies or products in a non-infringing manner. For example, a third-party may develop a competitive product that provides benefits similar to one or more of our current or future product candidates but that has a different composition that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our current or future product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our current or future product candidates could be negatively affected, which would harm our business.

Furthermore, even if we are able to issue patents with claims of valuable scope in one or more jurisdictions, we may not be able to secure such claims in all relevant jurisdictions, or in a sufficient number to meaningfully reduce competition. Our competitors may be able to develop and commercialize their products, including products identical to ours, in any jurisdiction in which we are unable to obtain, maintain or enforce such patent claims. Furthermore, generic manufacturers may develop, seek approval for and launch generic versions of our products, and may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings.

We also intend to rely on regulatory exclusivity for protection of our product candidates, if approved for commercial sale. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity or failure to obtain or to maintain the extent or

duration of such protections that we expect for our product candidates, if approved, could affect our decision on whether to market the products in a particular country or countries or could otherwise have an adverse impact on our revenue or results of operations.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, deadlines, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated if we fail to comply with these requirements. We may miss a filing deadline for patent protection on these inventions.

The USPTO and foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after issuance of any patent. In addition, periodic maintenance fees, renewal fees, annuity fees and/or various other government fees are required to be paid periodically. While an inadvertent lapse can, in some cases, be cured by payment of a late fee, or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market with similar or identical products or platforms, which could have a material adverse effect on our business prospects and financial condition.

If our trademarks and trade names for our products or company name are not adequately protected in one or more countries where we intend to market our products, we may delay the launch of product brand names, use different trademarks or tradenames in different countries, or face other potentially adverse consequences to building our product brand recognition.

Our trademarks or trade names may be challenged, infringed, diluted, circumvented or declared generic or determined to be infringing on other marks. We intend to rely on both registration and common law protection for our trademarks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During the trademark registration process, we may receive Office Actions from the USPTO or from comparable agencies in foreign jurisdictions objecting to the registration of our trademark. Although we would be given an opportunity to respond to those objections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and/or to seek the cancellation of registered trademarks. Opposition or cancellation proceedings may be filed against our trademark applications or registrations, and our trademark applications or registrations may not survive such proceedings. If we are unable to obtain a registered trademark or establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

If we are unable to adequately protect and enforce our trade secrets, our business and competitive position would be harmed.

In addition to the protection afforded by patents we may own or in-license, we seek to rely on trade secret protection, confidentiality agreements, and license agreements to protect proprietary know-how that may not be patentable, processes for which patents are difficult to enforce and any other elements of our product discovery and development processes that involve proprietary know-how, information or technology that may not be covered by patents. Although we require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, trade secrets can be difficult to protect and we have limited control over the protection of trade secrets used by our collaborators and suppliers. We cannot be certain that we have or will obtain these agreements in all circumstances and we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary information.

Moreover, any of these parties might breach the agreements and intentionally or inadvertently disclose our trade secret information and we may not be able to obtain adequate remedies for such breaches. In addition, competitors may otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights and trade secrets to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the U.S. and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, financial condition, results of operations and future prospects.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access

to our trade secrets or disclose our technology. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us.

Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary technology by third parties. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. Although we require all of our employees to assign their inventions to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be ineffective or breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We may initiate, become a defendant in or otherwise become party to lawsuits to protect or enforce our intellectual property rights, which could be expensive, time-consuming, and unsuccessful.

Competitors may infringe any patents we may own or in-license. In addition, any patents we may own or in-license also may become involved in inventorship, priority, validity or unenforceability disputes. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. In addition, in an infringement proceeding, a court may decide that one or more of any patents we may own or in-license is not valid or is unenforceable or that the other party's use of our technology that may be patented falls under the safe harbor to patent infringement under 35 U.S.C. § 271(e)(1). There is also the risk that, even if the validity of these patents is upheld, the court may refuse to stop the other party from using the technology at issue on the grounds that any patents we may own or in-license do not cover the technology in question or that such third-party's activities do not infringe our patent applications or any patents we may own or in-license. An adverse result in any litigation or defense proceedings could put one or more of any patents we may own or in-license at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, patient support or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of its greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

Post-grant proceedings provoked by third parties or brought by or before the USPTO or other patent granting authority may be necessary to determine the validity or priority of inventions with respect to our patent applications or any patents we may own or in-license. These proceedings are expensive and an unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to us from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. In addition to potential USPTO post-grant proceedings, we may become a party to patent opposition proceedings in the EPO, or similar proceedings in other foreign patent offices or courts where our patents may be challenged. The costs of these proceedings could be substantial, and may result in a loss of scope of some claims or a loss of the entire patent. An unfavorable result in a post-grant challenge proceeding may result in the loss of our right to exclude others from practicing one or more of our inventions in the relevant country or jurisdiction, which could have a material adverse effect on our business. Litigation or post-grant proceedings within patent offices may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the U.S.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

We may not be able to detect infringement against any patents we may own or in-license. Even if we detect infringement by a third party of any patents we may own or in-license, we may choose not to pursue litigation against or settlement with the third party. If we later sue such third-party for patent infringement, the third-party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us to enforce any patents we may own or in-license against such third party.

Intellectual property litigation and administrative patent office patent validity challenges in one or more countries could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, patient support or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. As noted above, some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace, including compromising our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development collaborations that would help us commercialize our current or future product candidates, if approved. Any of the foregoing events would harm our business, financial condition, results of operations and prospects.

We may be subject to damages or settlement costs resulting from claims that we or our employees have violated the intellectual property rights of third parties, or are in breach of our agreements. We may be accused of, allege or otherwise become party to lawsuits or disputes alleging wrongful disclosure of third-party confidential information by us or by another party, including current or former employees, contractors or consultants. In addition to diverting attention and resources to such disputes, such disputes could adversely impact our business reputation and/or protection of our proprietary technology.

The intellectual property landscape relevant to our product candidates and programs is crowded, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business. Our commercial success depends upon our ability to develop, manufacture, market and sell our current and future product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including derivation, interference, reexamination, *inter partes* review and post grant review proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We or any of our current or future licensors or strategic partners may be party to, exposed to, or threatened with, future adversarial proceedings or litigation by third parties having patent or other intellectual property rights alleging that our current or future product candidates and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights. We cannot assure you that our current or future product candidates and other technologies that we have developed, are developing or may develop in the future do not or will not infringe, misappropriate or otherwise violate existing or future patents or other valid intellectual property rights owned by third parties. For example, many of our employees were previously employed at other biotechnology or pharmaceutical companies. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's former employer. We may also be subject to claims that patents and applications we have filed to protect inventions of our employees, consultants and advisors, even those related to one or more of our current or future product candidates, are rightfully owned by their former or concurrent employer. Litigation may be necessary to defend against these claims.

While certain activities related to development and clinical testing of our current or future product candidates may be subject to safe harbor of patent infringement, such as under 35 U.S.C. §271(e)(1), upon receiving regulatory approval for such candidates we or any of our current or future licensors or strategic partners may immediately become party to, exposed to or threatened with, future adversarial proceedings or litigation by third parties having patent or other intellectual property rights alleging that such product candidates infringe, misappropriate or otherwise violate their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our current or future product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our current or future product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their

methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our current or future product candidates, technologies or methods.

If a third party claims that we infringe, misappropriate or otherwise violate its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement, misappropriation and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business and may impact our reputation;
- substantial damages for infringement, misappropriation or other violations, which we may have to pay if a court decides that the product candidate or technology at issue infringes, misappropriates or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our current product candidates, including bitopertin, DISC-0974 and DISC-3405, or future product candidates, or from using our proprietary technologies, unless the third-party licenses its product rights to us, which we are not required to do, on commercially reasonable terms or at all;
- if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products, or the license to us may be non-exclusive, which would permit third parties to use the same intellectual property to compete with us;
- redesigning our current or future product candidates or processes so they do not infringe, misappropriate or violate third-party intellectual property rights, which may not be possible or may require substantial monetary expenditures and time; and
- there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects. The occurrence of any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

We may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in an ex-parte re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third party's patent in patent opposition proceedings in the EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our current or future product candidates or proprietary technologies.

Third parties may assert that we are employing their proprietary technology without authorization. Patents issued in the U.S. by law enjoy a presumption of validity that can be rebutted in U.S. courts only with evidence that is "clear and convincing," a heightened standard of proof. There may be issued third-party patents of which we are currently unaware with claims to compositions, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our current or future product candidates. Patent applications can take many years to issue. In addition, because some patent applications in the U.S. may be maintained in secrecy until the patents are issued, patent applications in the U.S. and many foreign jurisdictions are typically not published until 18 months after their earliest priority filing date, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications covering our current or future product candidates or technology. If any such patent applications issue as patents, and if such patents have priority over our patent applications or patents we may own or in-license, we may be required to obtain rights to such patents owned by third parties which may not be available on commercially reasonable terms or at all, or may only be available on a non-exclusive basis. There may be currently pending third-party patent applications which may later result in issued patents that our current or future product candidates may infringe. It is also possible that patents owned by third parties of which we are aware, but which we do not believe are relevant to our current or future product candidates or other technologies, could be found to be infringed by our current or future product candidates or other technologies. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Moreover, we may fail to identify relevant patents or incorrectly conclude that a patent is invalid, not

enforceable, exhausted or not infringed by our activities. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our current or future product candidates, molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our current or future product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be nonexclusive, thereby giving our competitors access to the same technologies licensed to us.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our current or future product candidates. Defense of these claims, regardless of their merit, could involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement, misappropriation or other violation against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need or may choose to obtain licenses from third parties to advance our research or allow commercialization of our current or future product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our current or future product candidates, which could harm our business significantly.

We may be unable to obtain patent or other intellectual property protection for our current or future product candidates or our future products, if any, in all jurisdictions throughout the world, and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection.

We may not be able to pursue patent coverage of our current or future product candidates in all countries. Filing, prosecuting and defending patents on current or future product candidates in all countries throughout the world would be prohibitively expensive, and intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the U.S., or from selling or importing products made using our inventions in and into the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the U.S. These products may compete with our current or future product candidates and in jurisdictions where we do not have any issued patents, our patent applications or other intellectual property rights may not be effective or sufficient to prevent them from competing. Much of our patent portfolio is at the very early stage. We will need to decide whether and in which jurisdictions to pursue protection for the various inventions in our portfolio prior to applicable deadlines.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biopharmaceutical products and/or methods of using biopharmaceutical products, which could make it difficult for us to stop the infringement of any patents we may own or in-license or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce any rights we may have in our patent applications or any patents we may own or in-license in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put any patents we may own or in-license at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents we may own or license that are relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

We may not obtain or grant licenses or sublicenses to intellectual property rights in all markets on equally or sufficiently favorable terms with third parties.

It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. The licensing of third-party intellectual property rights is a competitive area, and more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. More established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to license such technology, or if we are forced to license such technology on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected current or future product candidates, which could materially harm our business, and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. Any of the foregoing could harm our competitive position, business, financial condition, results of operations and prospects.

If we fail to comply with our obligations in any agreements under which we may license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are party to license agreements with Roche, AbbVie and Mabwell and we may from time to time in the future be party to other license and collaboration agreements with third parties to advance our research or allow commercialization of current or future product candidates. Such agreements may impose numerous obligations, such as development, diligence, payment, commercialization, funding, milestone, royalty, sublicensing, insurance, patent prosecution, enforcement and other obligations on us and may require us to meet development timelines, or to exercise commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. See “Business - Collaborations and License Agreement” for more information regarding our license agreements with Roche, AbbVie and Mabwell. In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technologies covered by these license agreements.

Any termination of these licenses, or if the underlying patents fail to provide the intended exclusivity, could result in the loss of significant rights and could harm our ability to commercialize our current or future product candidates, and competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our current or future product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe, misappropriate or otherwise violate intellectual property rights of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our current or future product candidates, and what activities satisfy those diligence obligations;
- the priority of invention of any patented technology; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners.

In addition, the agreements under which we may license intellectual property or technology from third parties are likely to be complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we may license prevent or impair our ability to maintain future licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected current or future product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

Any granted patents we may own or in-license covering our current or future product candidates or other valuable technology could be narrowed or found invalid or unenforceable if challenged in court or before administrative bodies in the U.S. or abroad,

including the USPTO and the EPO. A patent asserted in a judicial court could be found invalid or unenforceable during the enforcement proceeding. Administrative or judicial proceedings challenging the validity of our patents or individual patent claims could take months or years to resolve.

If we or our licensors or strategic partners initiate legal proceedings against a third party to enforce a patent covering one of our current or future product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of patentable subject matter, lack of written description, lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO that was material to patentability, or made a misleading statement, in the process of obtaining the patent during patent prosecution. Third parties may also raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, *inter partes* review, post grant review and equivalent proceedings in foreign jurisdictions (such as opposition proceedings). Such proceedings could result in revocation or amendment to our patent applications or any patents we may own or in-license in such a way that they no longer cover our current or future product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, any rights we may have from our patent applications or any patents we may own or in-license, allow third parties to commercialize our current or future product candidates or other technologies and compete directly with us, without payment to us or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, we may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our or our current or future licensors' priority of invention or other features of patentability with respect to our patent applications and any patents we may own or in-license. Such challenges may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our current or future product candidates and other technologies. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we or our current or future licensing partners and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our current or future product candidates. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and current or future product candidates.

Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. If we are unsuccessful in any such proceeding or other priority or inventorship dispute, we may be required to obtain and maintain licenses from third parties, including parties involved in any such interference proceedings or other priority or inventorship disputes. Such licenses may not be available on commercially reasonable terms or at all, or may be non-exclusive. If we are unable to obtain and maintain such licenses, we may need to cease the development, manufacture, and commercialization of one or more of the current or future product candidates we may develop. The loss of exclusivity or the narrowing of our patent application claims could limit our ability to stop others from using or commercializing similar or identical technology and products. Any of the foregoing could have a material adverse effect on our business, results of operations, financial condition and prospects.

Changes in patent law could diminish the value of patents in general, thereby impairing our ability to protect our current or future product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the U.S. and other countries, including the Leahy-Smith America Invents Act, or Leahy-Smith Act, signed into law on September 16, 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a "first inventor to file" system. The first-inventor-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been

recent proposals for additional changes to the patent laws of the U.S. and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might subject us to infringement claims or adversely affect our ability to develop and market our current or future product candidates.

We cannot guarantee that any of our or our licensors' patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending patent application in the U.S. and abroad that is relevant to or necessary for the commercialization of our current or future product candidates in any jurisdiction. For example, U.S. patent applications filed before November 29, 2000 and certain U.S. patent applications filed after that date that will not be filed outside the U.S. remain confidential until patents issue. As mentioned above, patent applications in the U.S. and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our current or future product candidates could have been filed by third parties without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our current or future product candidates or the use of our current or future product candidates. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope and/or validity of a patent or a pending application may be incorrect, which may negatively impact our ability to market our current or future product candidates. We may incorrectly determine that our current or future product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the U.S. or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our current or future product candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our current or future product candidates.

If we fail to identify and correctly interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay damages, which may be significant, we may be temporarily or permanently prohibited from commercializing any of our current or future product candidates that are held to be infringing. We might, if possible, also be forced to redesign current or future product candidates so that we no longer infringes the third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business and could adversely affect our business, financial condition, results of operations and prospects.

Intellectual property rights do not guarantee commercial success of current or future product candidates or other business activities. Numerous factors may limit any potential competitive advantage provided by our intellectual property rights.

The degree of future protection afforded by our intellectual property rights, whether owned or in-licensed, is uncertain because intellectual property rights have limitations, and may not adequately protect our business, provide a barrier to entry against our competitors or potential competitors, or permit us to maintain our competitive advantage. Moreover, if a third party has intellectual property rights that cover the practice of our technology, we may not be able to fully exercise or extract value from our intellectual property rights. The following examples are illustrative:

- patent applications that we own or may in-license may not lead to issued patents;
- patents, should they issue, that we may own or in-license, may not provide us with any competitive advantages, may be narrowed in scope, or may be challenged and held invalid or unenforceable;
- others may be able to develop and/or practice technology, including compounds that are similar to the chemical compositions of our current or future product candidates, that is similar to our technology or aspects of our technology but that is not covered by the claims of any patents we may own or in-license, should any patents issue;
- third parties may compete with us in jurisdictions where we do not pursue and obtain patent protection;
- we, or our current or future licensors or collaborators, might not have been the first to make the inventions covered by a patent application that we own or may in-license;
- we, or our current or future licensors or collaborators, might not have been the first to file patent applications covering a particular invention;

- others may independently develop similar or alternative technologies without infringing, misappropriating or otherwise violating our intellectual property rights;
- our competitors might conduct research and development activities in the U.S. and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights, and may then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not be able to obtain and/or maintain necessary licenses on reasonable terms or at all;
- third parties may assert an ownership interest in our intellectual property and, if successful, such disputes may preclude us from exercising exclusive rights, or any rights at all, over that intellectual property;
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third-party may subsequently file a patent covering such trade secrets or know-how;
- we may not be able to maintain the confidentiality of our trade secrets or other proprietary information;
- we may not develop or in-license additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Risks Related to Government Regulation

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants regulatory approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In short, the foreign regulatory approval process involves all of the risks associated with FDA approval. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we may intend to charge for our products will also be subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable regulatory approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

We may seek priority review designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily result in an expedited regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

We may seek orphan drug designation for certain of our product candidates, and we may be unsuccessful or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We have received orphan drug designation from the FDA for bitopertin for the treatment of EPP and for DISC-3405 for the treatment of PV. As part of our business strategy, we may seek orphan drug designation for certain of our product candidates and indications, as appropriate. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is a product intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population of 200,000 or more in the United States where there is no reasonable expectation that the cost of developing the product will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers.

Similarly, in the European Union, the European Commission, upon the recommendation of the EMA's Committee for Orphan Medicinal Products, grants orphan designation in respect of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting no more than 5 in 10,000 persons in the European Union. Additionally, designation may be granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the product in the European Union would generate sufficient return to justify the necessary investment in developing the product. In each case, there must be no satisfactory method of diagnosis, prevention, or treatment of the applicable condition which is authorized for marketing in the European Union (or, if such a method exists, the applicable product would be of significant benefit to those affected by the condition). In the European Union, orphan designation entitles a party to financial incentives such as reduction of fees or fee waivers. The European Committee for Orphan Medical Products adopted a positive opinion on Orphan Designation for bitopertin for treatment of EPP.

Generally, if a product with an orphan designation subsequently receives the first regulatory approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product and indication for that time period, except in limited circumstances. The applicable period is seven years in the United States and ten years in the European Union. The European Union market exclusivity period can be reduced to six years if, at the end of the fifth year, it is determined that a product no longer meets the criteria for orphan designation, including if the product is sufficiently profitable so that market exclusivity is no longer justified.

Even if we obtain orphan drug exclusivity for one of our product candidates, that exclusivity may not effectively protect our product candidate from competition because different products can be approved for the same condition. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition or if another product with the same active moiety is determined to be safer, more effective, or represents a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a product nor gives the product any advantage in the regulatory review or approval process. While we may seek orphan drug designation for our product candidates, we may never receive such designations. Even if we do receive such designations, there is no guarantee that we will enjoy the benefits of those designations.

The FDA may further reevaluate its regulations and policies under the Orphan Drug Act. We do not know if, when or how the FDA may change its orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

We have received rare pediatric disease designation for bitopertin. However, a marketing application for bitopertin, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher.

In May 2024, we received rare pediatric disease designation for bitopertin in patients with EPP and XLP. The FDA defines "rare pediatric disease" as a (i) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect ages from birth to 18 years, including age groups often called neonates, infants, children and adolescents; and (ii) a rare disease or condition within the meaning of the Orphan Drug Act. Designation of a product candidate as a product for a rare pediatric disease does not guarantee that a marketing application for such product candidate will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. The FDA may determine that a marketing application for bitopertin, if approved, does not meet the eligibility criteria for a priority review voucher.

Under the current statutory sunset provisions, the FDA may only award a priority review voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug or biologic that is the subject of such application, and that designation was granted by December 20, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. As such, if we do not obtain approval of a marketing application for bitopertin in patients with EPP and XLP on or before September 30, 2026, we may not receive a priority review voucher. However, it is

possible that the FDA's authority to grant rare pediatric disease designation or award rare pediatric disease priority review vouchers will be further extended by Congress.

A breakthrough therapy designation and fast track designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review or approval process, and each designation does not increase the likelihood that any of our product candidates will receive regulatory approval in the United States.

We may seek a breakthrough therapy designation for certain of our product candidates. A breakthrough therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug or biologic may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Products designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates are designated as breakthrough therapies, the FDA may later withdraw the designation if it determines that such product candidates no longer meet the conditions for such designation.

We were granted fast track designation by the FDA for DISC-3405 for the treatment of PV in September 2023 and for DISC-0974 for the treatment of anemia in non-dialysis dependent chronic kidney disease in February 2024, and we may seek fast track designation for certain of our product candidates. If a drug or biologic is intended for the treatment of a serious or life-threatening condition and the drug or biologic demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for fast track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, the FDA may disagree and instead decide not to grant it. Even if we do receive fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation no longer meets the conditions for such designation. Fast track designation alone does not guarantee qualification for the FDA’s priority review procedures.

We may seek approval from the FDA for bitopertin or any of our other current or future product candidates under the Accelerated Approval Program. If we are not able to use such program, we may be required to conduct additional clinical trials beyond those that are contemplated, which would increase the expense of obtaining, and delay the receipt of, necessary marketing approvals, if we receive them at all. In addition, even if the Accelerated Approval Program is available to us, it may not lead to expedited approval of our product candidates, or approval at all.

We may seek accelerated approval of bitopertin or any of our other current or future product candidates. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. It is possible that at the time of submission of a marketing application, the FDA may determine that our product candidate is not eligible for accelerated approval or that accelerated approval is not warranted. Moreover, FDA may revise how it implements accelerated approval, which could negatively affect the development of our current or future product candidates.

As a condition of approval, the FDA generally requires that a sponsor of a drug or biologic receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence. Under FDORA, the FDA is permitted to require, that a post-approval confirmatory trial or trials be underway prior to approval or within a specified time period after the date accelerated approval is granted. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such trials, including progress toward enrollment targets, and the FDA must promptly post this information publicly. FDORA also gives the FDA increased authority to withdraw approval of a drug or biologic granted accelerated approval on an expedited basis if the sponsor fails to conduct confirmatory studies in a timely manner, send the necessary updates to the FDA, or if such post-approval trials fail to verify the product’s predicted clinical benefit. Under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory trial or submit timely reports to the agency on their progress. In addition, the FDA currently requires, unless the sponsor is otherwise informed by the agency, submission of launch promotional materials during the pre-approval period, which could adversely impact the timing of the commercial launch of the product.

If we are not able to obtain accelerated approval for a product candidate, we may be required to conduct additional clinical trials beyond those that are contemplated, which would increase the expense of obtaining, and delay the receipt of, full FDA approval, if received at all.

In November 2024, we announced that, based on discussions with the FDA, reduction in protoporphyrin IX, or PPIX, may be sufficient to serve as a surrogate endpoint supportive of submitting an NDA for bitopertin in EPP and XLP under the FDA's Accelerated Approval Program based on our existing data. In January 2025, we announced that we had aligned with the FDA on the design of our planned APOLLO post-marketing confirmatory trial and that we plan to initiate the trial in mid-2025 and submit an NDA for accelerated approval of bitopertin in EPP and XLP in the second half of 2025. However, the FDA could change its position regarding the sufficiency of PPIX as a surrogate endpoint or the design of our APOLLO clinical trial. Further, even if the FDA continues to agree that reduction of PPIX may be sufficient to serve as a surrogate endpoint supportive of submitting an NDA for accelerated approval of bitopertin in EPP and XLP and remains aligned with the design of APOLLO as a post-marketing confirmatory trial, the FDA may not accept for filing any NDA we submit for accelerated approval for bitopertin, may not grant this approval on a timely basis, or may not grant approval at all. Receiving accelerated approval does not provide assurance of ultimate full FDA approval. Even if the Accelerated Approval Program does result in a faster approval process, there is no guarantee that we will be able to begin commercialization of our product at the time of such approval, that the APOLLO trial will confirm and verify clinical benefit, or that we will be otherwise able to maintain such approval.

If our drug product candidates or any of our future drug product candidates obtain regulatory approval, additional competitors could enter the market with generic versions of such products, which may result in a material decline in sales of our competing products.

Under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments to the Federal Food, Drug, and Cosmetic Act, or the FDCA, a company may file an abbreviated new drug application, or ANDA, seeking approval of a generic version of an approved innovator product. Under the Hatch-Waxman Amendments, a company may also submit an NDA under section 505(b)(2) of the FDCA that references the FDA's prior approval of the innovator product or preclinical studies and/or clinical trials that were not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. A 505(b)(2) NDA product may be for a new or improved version of the original innovator product. The Hatch-Waxman Amendments also provide for certain periods of regulatory exclusivity, which preclude FDA approval (or in some circumstances, FDA filing and review) of an ANDA or 505(b)(2) NDA. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the Orange Book. If there are patents listed in the Orange Book for the applicable, approved innovator product, a generic or 505(b)(2) applicant that seeks to market our product before expiration of the patents must include in their applications what is known as a "Paragraph IV" certification, challenging the validity or enforceability, or claiming non-infringement, of the listed patent or patents. Notice of the certification must be given to the patent owner and NDA holder and if, within 45 days of receiving notice, either the patent owner or NDA holder sues for patent infringement, approval of the ANDA or 505(b)(2) NDA is stayed for up to 30 months.

Accordingly, if any of our product candidates that are regulated as drugs are approved, competitors could file ANDAs for generic versions of these products or 505(b)(2) NDAs that reference our products. If there are patents listed for such drug products in the Orange Book, those ANDAs and 505(b)(2) NDAs would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. We cannot predict which, if any, patents in our current portfolio or patents we may obtain in the future will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents or the outcome of any such suit.

We may not be successful in securing or maintaining proprietary patent protection for products and technologies we develop or licenses, despite expending a significant amount of resources that could have been focused on other areas of our business. Moreover, if any of our owned or in-licensed patents that are listed in the Orange Book are successfully challenged by way of a Paragraph IV certification and subsequent litigation, the affected product could immediately face generic competition and our sales would likely decline rapidly and materially.

If approved, our investigational products regulated as biologics may face competition from biosimilars or interchangeable products approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, created an abbreviated approval pathway for biologic products that are biosimilar to or interchangeable with an FDA-licensed reference biologic product. Under the BPCIA, an application for a biosimilar or interchangeable product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar or interchangeable product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

We believe that any of our product candidates approved as a biologic product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our investigational medicines to be reference products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of litigation. Moreover, the extent to which a biosimilar or interchangeable product, once licensed, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biologic products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain regulatory approval for biosimilars or interchangeable products referencing our products, our products may become subject to competition from such biosimilars or interchangeable products, with the attendant competitive pressure and consequences.

The FDA, the EMA and other regulatory authorities may implement additional regulations or restrictions on the development and commercialization of our product candidates, and such changes can be difficult to predict.

The FDA, the EMA and regulatory authorities in other countries have each expressed interest in further regulating biotechnology products. Agencies at both the federal and state level in the United States, as well as the U.S. Congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates. Adverse developments in clinical trials of products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. These regulatory review agencies and committees and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory agencies and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.

If any of our product candidates are approved and we are found to have improperly promoted off-label uses of those products, we may become subject to significant liability. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, if approved. In particular, while the FDA permits the dissemination of truthful and non-misleading information about an approved product, a manufacturer may not promote a product for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees, corporate integrity agreements or permanent injunctions under which specified promotional conduct must be changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

Inadequate funding for the NIH, CMS, FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, including significant leadership, personnel, or policy changes, could prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

Currently, federal agencies in the U.S. are operating under a continuing resolution that is set to expire on March 14, 2025. Without appropriation of additional funding to federal agencies, our business operations related to our product development activities for the U.S. market could be impacted. The ability of the FDA to review and approve new products, NIH's ability to conduct and partner with industry on important research, and CMS's ability to operate efficiently can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. Disruptions at these agencies, including, for example, as a result of the freeze on federal funding announced in January 2025 and other restrictions, such as personnel reductions at agencies such as the FDA, may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies or may slow or stall planned or ongoing research, which would adversely affect our business.

For example, over the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown or a widespread freeze on federal funding occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, NIH to conduct research or provide grants, or other agencies to slow their work, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Healthcare reform measures may have a material adverse effect on our business and results of operations.

The U.S. and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the U.S. and global healthcare systems that could prevent or delay regulatory approval of our current or future product candidates or any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell a product for which we obtain regulatory approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements, (ii) additions or modifications to product labeling, (iii) the recall or discontinuation of our products or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. In the U.S., there have been and continue to be, on-going legislative initiatives to contain healthcare costs. For example, the Patient Protection and Affordable Care Act, or the ACA, as amended by the Health Care and Education Reconciliation Act of 2010, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program, and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, creates a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D, and provided incentives to programs that increase the federal government's comparative effectiveness research. Since then, the ACA risk adjustment program payment parameters have been updated annually.

Since the ACA's enactment, there have been numerous judicial, administrative, executive and legislative challenges to certain aspects of the ACA and we expect that there will be additional challenges and amendments to the ACA in the future. On June 17, 2021, for example, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. In addition, multiple executive orders have been issued that seek to reduce prescription drug costs. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the current administration may reverse or otherwise change these measures, both the current administration and Congress have indicated that they will continue to seek new measures to control drug costs. It is unclear how healthcare reform measures or other efforts, if any, to challenge repeal or replace the ACA, will impact our business.

In addition, other legislative and regulatory changes have been proposed and adopted in the United States since the ACA was enacted:

- The U.S. Budget Control Act of 2011, for example, included aggregate reductions of Medicare payments to providers of 2% per fiscal year that will remain in effect through 2032. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers will be further reduced starting in 2025 absent further legislation.

- The U.S. American Taxpayer Relief Act of 2012 further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.
- The U.S. American Rescue Plan Act of 2021, which eliminates the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, effective January 1, 2024.

There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs.

The Inflation Reduction Act of 2022, or the IRA, includes several provisions that may impact our business, depending on how various aspects of the IRA are implemented. Provisions that may impact our business include a $2,000 out-of-pocket cap for Medicare Part D beneficiaries, the imposition of new manufacturer financial liability on most drugs in Medicare Part D, permitting the U.S. government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition, requiring companies to pay rebates to Medicare for drug prices that increase faster than inflation, and delay until January 1, 2032 the implementation of the HHS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The implementation of the IRA is currently subject to ongoing litigation challenging the constitutionality of the IRA's Medicare drug price negotiation program. CMS has announced the results of the first round of negotiated prices between CMS and participating drug manufacturers for the ten selected drugs under the IRA's Medicare drug price negotiation program. These prices go into effect in 2026. CMS has also announced the next 15 drugs selected for the IRA's Medicare drug price negotiation program. It is unclear whether ongoing litigation will be successful in challenging the constitutionality of the IRA's Medicare drug price negotiation program and, if so, may provide a mechanism to block the implementation of the program. The effects of the IRA on our business and the healthcare industry in general is not yet known.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing.

We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, or the frequency with which any such product candidate is prescribed or used, which could result in reduced demand for our current or future product candidates or additional pricing pressures. In particular any policy changes through CMS as well as local state Medicaid programs could have a significant impact on our business.

Our revenue prospects could be affected by changes in healthcare spending and policy in the U.S. and abroad. We operate in a highly regulated industry and new laws, regulations or judicial decisions, or new interpretations of existing laws, regulations or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could negatively impact our business, operations and financial condition.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future, including repeal, replacement or significant revisions to the ACA. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our current or future product candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and reimbursement approval for a product;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and

- the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

Our relationships with customers, healthcare providers, physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm and diminished future profits and earnings.

Although we do not currently have any products on the market, once we begin commercializing our product candidates, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain regulatory approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our product candidates for which we obtain regulatory approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations are subject to civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment of up to ten years, and exclusion from government healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act or federal civil money penalties. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers, on the one hand, and prescribers, purchasers and formulary managers, on the other. The HHS, Office of Inspector General, or OIG, heavily scrutinizes relationships between pharmaceutical companies and persons in a position to generate referrals for or the purchase of their products, such as physicians, other healthcare providers, and pharmacy benefit managers, among others. However, there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;
- the federal civil and criminal false claims and civil monetary penalties laws, including the federal False Claims Act, or FCA, which imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false of fraudulent claim for purposes of the False Claims Act. The federal False Claims Act also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the federal False Claims Act and to share in any monetary recovery;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program (e.g. public or private), or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal physician payment transparency requirements, sometimes referred to as the "Sunshine Act" under the ACA, which require manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program to report to HHS information related to transfers of value made to physicians, nurse practitioners, certified nurse anesthetists, physician assistants, clinical nurse specialists, and certified nurse midwives as well as teaching hospitals. Manufacturers are also required to disclose ownership and investment interests held by physicians and their immediate family members;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- federal price reporting laws, which require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products.

We are also subject to state and foreign equivalents of each of the healthcare laws and regulations described above, among others, some of which may be broader in scope and may apply regardless of the payor. Many U.S. states have adopted laws similar to the federal Anti-Kickback Statute and False Claims Act, and may apply to our business practices, including, but not limited to, research, distribution, sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental payors, including private insurers. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and/or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state and require the registration of pharmaceutical sales representatives. State and foreign laws, including for example the European Union General Data Protection Regulation, also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties. Finally, there are state and foreign laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

In the U.S., to help patients afford our approved product, we may utilize programs to assist them, including patient assistance programs and co-pay coupon programs for eligible patients. Government enforcement agencies have shown increased interest in pharmaceutical companies' product and patient assistance programs, including reimbursement support services, and a number of investigations into these programs have resulted in significant civil and criminal settlements. In addition, at least one insurer has directed its network pharmacies to no longer accept co-pay coupons for certain specialty drugs the insurer identified. Our co-pay coupon programs could become the target of similar insurer actions. In addition, in November 2013, the CMS issued guidance to the issuers of qualified health plans sold through the ACA's marketplaces encouraging such plans to reject patient cost-sharing support from third parties and indicating that the CMS intends to monitor the provision of such support and may take regulatory action to limit it in the future. The CMS subsequently issued a rule requiring individual market qualified health plans to accept third-party premium and cost-sharing payments from certain government-related entities. In September 2014, the OIG of the HHS issued a Special Advisory Bulletin warning manufacturers that they may be subject to sanctions under the federal anti-kickback statute and/or civil monetary penalty laws if they do not take appropriate steps to exclude Part D beneficiaries from using co-pay coupons. Accordingly, companies exclude these Part D beneficiaries from using co-pay coupons. It is possible that changes in insurer policies regarding co-pay coupons and/or the introduction and enactment of new legislation or regulatory action could restrict or otherwise negatively affect these patient support programs, which could result in fewer patients using affected products, and therefore could have a material adverse effect on our sales, business, and financial condition.

Third party patient assistance programs that receive financial support from companies have become the subject of enhanced government and regulatory scrutiny. The OIG has established guidelines that suggest that it is lawful for pharmaceutical manufacturers to make donations to charitable organizations who provide co-pay assistance to Medicare patients, provided that such organizations, among other things, are bona fide charities, are entirely independent of and not controlled by the manufacturer, provide aid to applicants on a first-come basis according to consistent financial criteria and do not link aid to use of a donor's product. However, donations to patient assistance programs have received some negative publicity and have been the subject of multiple government enforcement actions, related to allegations regarding their use to promote branded pharmaceutical products over other less costly alternatives. Specifically, in recent years, there have been multiple settlements resulting out of government claims challenging the legality of their patient assistance programs under a variety of federal and state laws. It is possible that we may make grants to independent charitable foundations that help financially needy patients with their premium, co-pay, and co-insurance obligations. If we choose to do so, and if we or our vendors or donation recipients are deemed to fail to comply with relevant laws, regulations or evolving government guidance in the operation of these programs, we could be subject to damages, fines, penalties, or other criminal, civil or administrative sanctions or enforcement actions. We cannot ensure that our compliance controls, policies, and procedures will be sufficient to protect against acts of our employees, business partners or vendors that may violate the laws or regulations of the jurisdictions in which we operate. Regardless of whether we have complied with the law, a government investigation could impact our business practices, harm our reputation, divert the attention of management, increase our expenses and reduce the availability of foundation support for our patients who need assistance.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have

recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including anticipated activities to be conducted by our sales team, were to be found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, the exclusion from participation in federal and state government funded healthcare programs, such as Medicare and Medicaid, reputational harm, and the curtailment or restructuring of our operations. It may also subject us to additional reporting obligations and oversight, if we become subject to a corporate integrity agreement, deferred prosecution agreement, or other agreement to resolve allegations of non-compliance with these laws. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to similar criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

Compliance with U.S. and global privacy and security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. We possess and process sensitive information, including patient health information. In the U.S., there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including health information privacy laws, security breach notification laws and consumer protection laws. These federal and state laws, in many cases, are not preempted by HIPAA and may be subject to varying interpretations by the courts and government agencies. These varying interpretations can create complex compliance issues for us and our partners and potentially expose us to additional expense, adverse publicity and liability, any of which could adversely affect our business. There is ongoing concern from privacy advocates, regulators and others regarding data privacy and security issues, and the number of jurisdictions with data privacy and security laws has been increasing. Also, there are ongoing public policy discussions regarding whether the standards for de-identification, anonymization or pseudonymization of health information are sufficient, and the risk of re-identification sufficiently small, to adequately protect patient privacy. We expect that there will continue to be new proposed and amended laws, regulations and industry standards concerning privacy, data protection and information security in the United States. For example, California enacted the California Consumer Privacy Act, or CCPA, a comprehensive privacy law that broadly defines personal information, gives California residents expanded rights to access and delete their personal information, and places stringent privacy and security obligations on businesses covered by the law, including obligations to provide detailed disclosures to California consumers about their data collection, use and sharing practices, and to provide such consumers with ways to opt out of certain sales or transfers of personal information. It also provides for civil penalties for violations, and allows for a private right of action for data breaches that is expected to increase data breach litigation. In addition, the CCPA was amended by the California Privacy Rights Act, or CPRA, which as of January 1, 2023, significantly modified the CCPA by expanding consumer rights with respect to certain sensitive information, and creating a new state agency that is vested with authority to implement and enforce the CCPA.

Comprehensive laws similar to the CCPA have been passed in numerous other states outside of California. These laws are substantially similar in scope and contain many of the same requirements and exceptions as the CCPA, including a general exemption for clinical trial data and limited obligations for entities regulated by HIPAA. However, we cannot yet determine the full impact these laws or other such future laws, regulations and standards may have on our current or future business. Any of these laws may broaden their scope in the future, and similar laws have been proposed on both a federal level and in more than half of the states in the U.S. In addition, a number of other states have proposed new privacy laws, some of which are similar to the above discussed recently passed laws. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. Furthermore, in addition to comprehensive privacy laws, certain states have enacted laws to focus on particular more limited privacy laws. For example, the state of Washington has passed a law to protect medical and health information not subject to HIPAA. This law has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain specific types of information. For example, a small number of states have passed laws that regulate biometric information. The existence of comprehensive privacy laws in different states in the country would make our compliance obligations more complex and costly and may increase the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. These various privacy and security laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. State laws are changing rapidly and there have been proposals for a new comprehensive federal data privacy law to which we may likely become subject, if enacted.

The collection, use, storage, disclosure, transfer or other processing of personal data regarding individuals in the EEA and UK, including personal health data, is subject to the EU General Data Protection Regulation 2016/679, or EU GDPR, with respect to individuals in the EEA and the UK General Data Protection Regulation (following the incorporation of the EU GDPR into UK law), or UK GDPR, with respect to individuals in the UK, and together with the EU GDPR, GDPR. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to having a legal basis or condition for processing personal data, stricter requirements relating to processing sensitive data (such as health data), where required by GDPR obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, requiring data protection impact assessments for high risk processing and taking certain measures when engaging third-party processors. The GDPR permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million (£17.5 million for the UK) or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. The GDPR increased our responsibility and liability in relation to personal data that we process where such processing is subject to the GDPR, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, including as implemented by individual countries. Compliance with the GDPR is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with our European activities.

The GDPR provides that EEA Member States may make their own further laws and regulations in relation to the processing of genetic, biometric or health data, which could result in differences between Member States, limit our ability to use and share personal data or could cause our costs to increase, and harm our business and financial condition.

The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA and UK not deemed adequate for the transfer of such personal data by competent data protection authorities, or third countries, including the United States in certain circumstances unless a derogation exists or we may need to incorporate a GDPR transfer mechanism (such as the European Commission approved standard contractual clauses, or SCCs or the UK International Data Transfer Addendum, or IDTA) into our agreements with third parties to govern such transfers of personal data and carry out transfer impact assessments. Further, the European Union and United States have adopted its adequacy decision for the EU-U.S. Data Privacy Framework, or the Framework, which entered into force on July 11, 2023. This Framework provides that the protection of personal data transferred between the European Union and the United States is comparable to that offered in the European Union. This provides a further avenue to ensuring transfers to the United States are carried out in line with GDPR. There has been an extension to the Framework to cover UK transfers to the United States. The Framework could be challenged like its predecessor frameworks. The international transfer obligations under the EEA and UK data protection regimes will require effort and cost and may result in us needing to make strategic considerations around where EEA/UK personal data is located and which service providers we can utilize for the processing of EEA/UK personal data.

Although the UK is regarded as a third country under the EU GDPR, the European Commission has issued an “Adequacy Decision” recognizing the UK as providing adequate protection under the EU GDPR and, therefore, transfers of personal data subject to the EU GDPR to the UK remain unrestricted. The UK government has confirmed that personal data transfers from the

UK to the EEA remain free flowing. The UK Government introduced the Data Protection and Digital Information Bill, which failed in the UK legislative process. A new Data (Use and Access) Bill, or the UK Bill, has been introduced into parliament. If passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EU data protection regime and threaten the UK Adequacy Decision from the EU Commission. This may lead to additional compliance costs and could increase our overall risk.

In the EEA, the NIS 2 Directive, or NIS 2, is replacing the cybersecurity legal framework under the current NIS framework, aiming to ensure a high level of cybersecurity in the region. NIS 2 expands on the scope of the current NIS framework, extending to additional sectors and expanding the list of in-scope healthcare organisations providing services in the EEA, including to certain providers engaged in research and development of medicinal products. To the extent applicable, the new regime imposes direct obligations on management regarding compliance with NIS 2, requiring covered organisations to put in place certain cyber risk management measures, and strengthens incident reporting requirements and provides supervisory authorities with greater oversight. The majority of these obligations will not come into force until national legislation implementing NIS 2 becomes effective in the relevant EU Member State. EU Member States had until October 17, 2024 to transpose NIS 2 into national legislation, although many countries have still not completed this process. As such, the cybersecurity regulatory landscape in the EU is currently fragmented and uncertain. To the extent we become subject to NIS 2, we may need to increase investment in our cybersecurity compliance programs. Under NIS 2, companies may be subject to administrative fines of up to the higher amount of €10 million or 2% of worldwide annual revenue.

All of these evolving compliance and operational requirements impose potentially significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. In addition, such requirements may require us to modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects. If we or third-party CDMOs, CROs or other contractors or consultants fail to comply with U.S. and international data protection laws and regulations, it could result in government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects about whom we or our potential collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business, financial condition, results of operations and prospects.

The use of new and evolving technologies, such as artificial intelligence, in our business may result in spending material resources and presents risks and challenges that can impact our business including by posing security and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability.

We may use and integrate artificial intelligence, or AI, into our business processes, and this innovation presents risks and challenges that could affect its adoption, and therefore our business. If we enable or offer solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability. The use of certain AI technology can give rise to intellectual property risks, including compromises to proprietary intellectual property and intellectual property infringement. To the extent we develop our own AI systems, then the risk of intellectual property infringement could arise from third party data sources being used to train our AI models, and from the output of AI systems reproducing or incorporating third party intellectual property rights, in each case without the right to do so. Further, a risk of our proprietary intellectual property rights being compromised through the use of AI could arise through third party vendors using our data to train their models and/or to generate output for other users of their systems. If our third party vendors use our data (which includes personal data) to train their AI models and/or to generate output for other users of their systems, this could also give rise to regulatory risks. Additionally, we expect to see increasing government and supranational regulation related to AI use and ethics, which may also significantly increase the burden and cost of research, development and compliance in this area. For example, the European Union's Artificial Intelligence Act, or the AI Act, the world's first comprehensive AI law, entered into force in August 2024 and, with some exceptions, becomes effective in August 2025. This legislation imposes significant obligations on providers and deployers of high risk AI systems, and encourages providers and deployers of AI systems to account for European Union ethical principles in their development and use of these systems. If we develop or use AI systems that are governed by the AI Act, it may necessitate ensuring higher standards of data quality, transparency, and human oversight, as well as adhering to specific and potentially burdensome and costly ethical, accountability, and administrative requirements. The rapid evolution of AI will require the application of significant resources to design, develop, test and maintain our products and services to help ensure that AI is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. Our vendors may in turn incorporate AI tools into their offerings, and the providers of these AI tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of AI, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. Any of these

effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain products outside of the United States and require us to develop and implement costly compliance programs.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Risks Relating to Employee Matters and Managing Growth

Our future success depends on our ability to retain key executives and experienced scientists and to attract, retain, and motivate qualified personnel.

We are highly dependent on many of our key employees and members of our executive management team as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment letter agreements with certain of our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees, including temporary loss due to illness, could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel.

In particular, we have experienced a very competitive hiring environment in the greater Boston area of Massachusetts, where we are headquartered. Many of the other pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to

high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success with which we can discover and develop product candidates and our business will be limited.

We may be unable to adequately protect our information systems and infrastructure from cyberattacks or security compromises, cybersecurity incidents or data breaches, which could result in the disclosure of confidential or proprietary information, including personal data, damage our reputation, and subject us to significant financial and legal exposure.

We rely on information technology systems and infrastructure that we or our third-party providers operate to process, transmit and store electronic information in our day-to-day operations. In connection with our product discovery efforts, we may collect and use a variety of personal data and other confidential and/or proprietary data, such as names, mailing addresses, email addresses, phone numbers and clinical trial information. A successful cyberattack could result in the theft, destruction or misuse of intellectual property, data, or other misappropriation of assets, or otherwise compromise our confidential or proprietary information and disrupt our operations. Cyberattacks generally are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyberattacks could include wrongful conduct by hostile foreign governments, insiders such as employees, contractors or other third-parties and industrial espionage, wire fraud and other forms of cyber fraud, the deployment of harmful malware, ransomware, denial-of-service, social engineering fraud (including phishing attacks) or other means to threaten or compromise the security, confidentiality, integrity and availability of systems and information. A successful cyberattack could cause serious negative consequences for us, including, without limitation, the disruption of operations, the misappropriation of protected information or confidential business information, including personal data, financial information, trade secrets, financial loss and the disclosure of corporate strategic plans.

Like other companies in our industry, we, and our third party vendors, have experienced, and will continue to experience, cybersecurity threats and incidents relating to our information technology systems and infrastructure. Although we devote resources designed to protect our information systems and infrastructure, we realize that cyberattacks are a threat, and there can be no assurance that our efforts will prevent or adequately address information security compromises, incidents or breaches that would result in business, legal, financial or reputational harm to it, or would have a material adverse effect on our results of operations and financial condition. Any failure to prevent or mitigate cybersecurity incidents, data breaches, compromises or other improper access to, use of, or disclosure of protected information, including our clinical data or patients' personal data could require us to notify impacted stakeholders (including affected individuals, regulators and investors) and result in significant liability through litigation and regulatory investigations and enforcement actions, including under state (e.g., state breach notification and consumer protection laws), federal (e.g., HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH")), and international law (e.g., the GDPR), and may cause a material adverse impact to our reputation, affect our ability to conduct new studies and potentially disrupt our business.

We rely on our third-party providers to implement effective security measures and identify and correct for any such failures, deficiencies, vulnerabilities, compromises, cybersecurity incidents or data breaches. We also rely on our employees and consultants to safeguard their security credentials and follow our policies and procedures regarding use and access of computers and other devices that may contain protected information and our sensitive information. If we or our third-party providers fail to maintain or protect our information technology systems, infrastructure and data integrity effectively or fail to anticipate, plan for or manage significant disruptions to our information technology systems and infrastructure, we or our third-party providers could have difficulty preventing, detecting and controlling such cyber-attacks, and any such attacks could result in the losses described above as well as disputes with physicians, patients and our partners, regulatory sanctions or penalties, increases in operating expenses, expenses or lost revenue or other adverse consequences, any of which could have a material adverse effect on our business, results of operations, financial condition, prospects and cash flows. Any failure by such third parties to prevent or adequately mitigate cybersecurity incidents, data breaches, compromises or other improper access to or disclosure of such information could have similarly adverse consequences for us. If we are unable to prevent or adequately mitigate the impact of such cybersecurity incidents, compromises or data breaches, we could be exposed to litigation and governmental investigations, inquiries, orders, penalties or fines, which could lead to a potential disruption to our business and financial penalties or losses. By way of example, the CCPA, which was modified by the CPRA, creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal data. The CCPA provides for civil penalties of up to $7,500 per violation, as well as a private right of action for data breaches that has, and is expected to continue to, increase the volume of data breach related litigation filed in California. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations. Further, although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or data breach.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of December 31, 2024, we had 84 full-time employees and no part-time employees. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly as we mature as a public company and in the

areas of product development, regulatory affairs and, in anticipation of the potential regulatory approval of bitopertin, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction.

General Risk Factors

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults, or non-performance by financial institutions or transactional counterparties, could adversely affect the Company's current and projected business operations and its financial condition and results of operations.

Events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank, or SVB, was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation, or FDIC, as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each placed into receivership. Although a statement by the Department of the Treasury, the Federal Reserve and the FDIC indicated that all depositors of SVB (such as our Company) would have access to all of their money after only one business day of closure, including funds held in uninsured deposit accounts, borrowers under credit agreements, letters of credit and certain other financial instruments with SVB, Signature Bank or any other financial institution that is placed into receivership by the FDIC may be unable to access undrawn amounts thereunder. Subsequent to these events, additional financial institutions have experienced similar failures and have been placed into receivership. It is possible that other banks will face similar difficulty in the future.

Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect the Company, the financial institutions with which the Company has credit agreements or arrangements directly, such as Hercules Capital, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. These factors could involve financial institutions or financial services industry companies with which the Company has financial or business relationships, but could also include factors involving financial markets or the financial services industry generally.

In addition, investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

Changes in tax law may adversely affect us or our investors.

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service, or IRS, and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. For example, under Section 174 of the Code, in taxable years beginning after December 31, 2021, expenses that are incurred for research and development in the U.S. will be capitalized and amortized, which may have an adverse effect on our cash flow. It cannot be predicted whether, when, in what form or with what effective dates tax laws, regulations and rulings may be enacted, promulgated or issued, which could result in an

increase in our or our stockholders' tax liability or require changes in the manner in which we operate in order to minimize or mitigate any adverse effects of changes in tax law. Prospective investors should consult their tax advisors regarding the potential consequences of changes in tax law on our business and on the ownership and disposition of our common stock.

Our future taxable income may be subject to certain limitations.

As of December 31, 2024, we had federal and state net operating loss carryforwards of $117.9 million and $133.8 million, respectively. Substantially all of the federal NOLs are not subject to expiration and the state NOLs begin to expire in 2037. As of December 31, 2024, we also had federal and state research and development tax credit carryforwards of $19.0 million and $2.8 million, respectively, which begin to expire in 2034. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under current law, unused U.S. federal and certain state net operating losses generated for tax years beginning after December 31, 2017 are not subject to expiration and may be carried forward indefinitely. Such U.S. federal net operating losses generally may not be carried back to prior taxable years, except that, net operating losses generated in 2019, 2020 and 2021 may be carried back to each of the five tax years preceding the tax years of such losses. For taxable years beginning after December 31, 2020, the deductibility of U.S. federal net operating losses generated for tax years beginning after December 31, 2017 is limited to 80% of our taxable income in any future taxable year. In addition, in general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses or tax credits, or NOLs or credits, to offset future taxable income or taxes. For these purposes, an ownership change generally occurs when one or more stockholders or groups of stockholders who each owns at least 5% of a corporation's stock increase their aggregate stock ownership by more than 50 percentage points over their lowest ownership percentage within a specified testing period. Our existing NOLs or credits may be subject to limitations arising from previous ownership changes, and if we undergo an ownership change after the merger, our ability to utilize NOLs or credits could be further limited by Sections 382 and 383 of the Code. In addition, future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the Code. Our NOLs or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or credits.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations. We can face serious consequences for violations.

Among other matters, U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations, which are collectively referred to as Trade Laws, prohibit companies and their employees, agents, CROs, legal counsel, accountants, consultants, contractors and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase in time. We currently engage, and expect to continue to engage, third parties for clinical trials and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals and we can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, in 2008, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets and the COVID-19 pandemic has caused significant volatility and uncertainty in U.S. and international markets. See "Risks Related to the Discovery and Development of Our Product Candidates-A pandemic, epidemic, or outbreak of an infectious or highly contagious disease may materially and adversely affect our business and financial results and could cause a disruption to the development of our product candidates." Interest rates in the U.S. have recently increased to levels not seen in decades. In addition, the impact of geopolitical tension, such as a deterioration in the bilateral relationship between the United States and China or an escalation in conflict between Russia and Ukraine and between Israel and Hamas, including any resulting sanctions, export controls or other restrictive actions, also could lead to disruption, instability and volatility in the global markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our products. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements or insider trading.

We are exposed to the risk that our employees, principal investigators, CROs and consultants may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate the regulations of the FDA and other regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities; healthcare fraud and abuse laws and regulations in the United States and abroad; or laws that require the reporting of financial information or data accurately. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Other activities subject to these laws include the improper use of information obtained in the course of clinical trials or creating fraudulent data in our preclinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourself or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

The market price of our common stock is expected to be volatile.

The market price of our common stock following the merger could be subject to significant fluctuations. Some of the factors that may cause the market price of our common stock to fluctuate include:

- results of clinical trials and preclinical studies of our product candidates, or those of our competitors or our existing or future collaborators;
- failure to meet or exceed financial and development projections we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- if we do not achieve the perceived benefits of the merger as rapidly or to the extent anticipated by financial or industry analysts;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions or market conditions in the pharmaceutical and biotechnology sectors;
- sales of securities by us or our securityholders in the future;
- if we fail to raise an adequate amount of capital to fund our operations and continued development of our product candidates;
- trading volume of our common stock;

- announcements by competitors of new commercial products, clinical progress or lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to precision medicine product candidates, including with respect to other products in such markets;
- the introduction of technological innovations or new therapies that compete with our products and services; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In addition, a recession, depression or other sustained adverse market event could materially and adversely affect our business and the value of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against such companies. Furthermore, market volatility may lead to increased shareholder activism if we experience a market valuation that activists believe is not reflective of our intrinsic value. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results and financial condition.

We have incurred and will continue to incur increased costs and increased demands upon management as a result of complying with the laws and regulations affecting public companies.

We will continue to incur significant legal, accounting and other expenses as a public company that we did not incur as a private company, including costs associated with public company reporting obligations under the Securities Exchange Act of 1934, as amended, or the Exchange Act. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote and will need to continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased and will continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on the board of directors or on board committees or to serve as executive officers, or to obtain certain types of insurance, including directors' and officers' insurance, on acceptable terms.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. As described further below, we have identified a material weakness in our internal control over financial reporting. Any testing by us conducted in connection with Section 404, or any testing by our independent registered public accounting firm, may reveal additional deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. To continue to achieve and maintain compliance with Section 404, we engage in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, from time to time we may not be able to conclude that our internal control over financial reporting is effective as required by Section 404, as is the case in this Annual Report on Form 10-K, due to the material weakness identified and described below. Additionally, the material weakness in our internal control over financial reporting has resulted in our management being unable to conclude, and any additional material weakness in our internal control over financial reporting may in the future result in our management being unable to conclude, that our disclosure controls and procedures were effective for the applicable period.

In addition, as we no longer qualify as a non-accelerated filer or an emerging growth company, we are required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. If we are

unable to maintain effective internal control over financial reporting, we may not have adequate, accurate or timely financial information, and our independent registered public accounting firm may issue a report that is adverse. A material weakness could result in a restatement of our financial statements, failure to meet our reporting obligations in a timely manner, the imposition of sanctions, including the inability of registered broker dealers to make a market in our common stock, or investigation by regulatory authorities. Any such action or other negative results caused by our inability to meet our reporting requirements or comply with legal and regulatory requirements or by disclosure of an accounting, reporting or control issue could adversely affect the trading price of our securities and our business. Ineffective internal control over financial reporting could also reduce our ability to obtain financing or could increase the cost of any financing we obtain. Any of these could, in turn, result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We have identified a material weakness in our internal controls over financial reporting and may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, which may result in material misstatements of our consolidated financial statements or cause us to fail to meet our periodic reporting obligations.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of a company's annual or interim financial statements will not be prevented or detected on a timely basis. We identified a material weakness in internal control related to a lack of design and maintenance of effective Information Technology General Controls, or ITGCs, over certain key financial IT systems. As a result, the related business process controls (specifically, the IT application controls and IT-dependent manual controls) that are dependent on the ineffective ITGCs, or that use information produced from the systems impacted by the ineffective ITGCs, were also ineffective. Although the material weakness identified above did not result in any material misstatements in our consolidated financial statements for the periods presented and there were no changes to previously released financial results, our management concluded that these control deficiencies constitute a material weakness and that our internal control over financial reporting was not effective as of December 31, 2024.

Our management, under the oversight of the Audit Committee of our Board of Directors and in consultation with outside advisors, has begun evaluating and implementing measures designed to remediate the material weakness. In particular, we are taking steps to remediate this material weakness by (i) developing and implementing additional training and awareness programs addressing ITGCs and policies, including educating control owners concerning the principles and requirements of each control, with a focus on user access; (ii) increasing the extent of oversight and verification checks included in the operation of user access and program change management controls and processes; (iii) deploying additional tools to support administration of user access and program change management; and (iv) enhancing quarterly management reporting on the remediation measures to the Audit Committee of the Board of Directors. The above controls need to operate for a sufficient period of time so that management can conclude that our controls are operating effectively. As such, the material weakness will not be considered remediated until management has concluded through the implementation of these remediation measures and additional testing that these controls are effective. Additionally, a material weakness in our internal control over financial reporting has resulted in our management being unable to conclude, and any additional material weakness in our internal control over financial reporting may in the future result in our management being unable to conclude, that our disclosure controls and procedures were effective for the applicable period.

We are designing and implementing new controls and measures to remediate this material weakness as noted above. However, we cannot assure you that the measures we are taking will be sufficient to remediate the material weakness or avoid the identification of additional material weaknesses in the future. Our failure to implement and maintain effective internal control over financial reporting could result in errors in our consolidated financial statements that could result in a restatement of our financial statements and could cause us to fail to meet our periodic reporting obligations, any of which could diminish investor confidence in us and cause a decline in the price of our common stock.

Provisions in our charter documents and under Delaware law could make an acquisition of us more difficult and may discourage any takeover attempts our stockholders may consider favorable, and may lead to entrenchment of management.

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws could delay or prevent changes in control or changes in management without the consent of the board of directors. These provisions will include the following:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- a prohibition on stockholder action by written consent, which means that all stockholder action must be taken at an annual or special meeting of the stockholders;

- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the Chief Executive Officer or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to the board of directors;
- a requirement that no member of the board of directors may be removed from office by stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of voting stock to amend any bylaws by stockholder action or to amend specific provisions of the certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, these provisions would apply even if we were to receive an offer that some stockholders may consider beneficial.

We will also be subject to the anti-takeover provisions contained in Section 203 of the DGCL, or Section 203. Under Section 203, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

Our certificate of incorporation and bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or other employees.

Our certificate of incorporation and bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty, any action asserting a claim against it arising pursuant to any provisions of the DGCL, our certificate of incorporation or our bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. The exclusive forum provision does not apply to actions arising under the Exchange Act. Our amended and restated bylaws also provide that the federal district courts of the United States of America will be the exclusive forum for the resolution of any complaint asserting a cause of action under the Securities Act. The provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in the certificate of incorporation and bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could materially and adversely affect our business, financial condition and results of operations.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings, if any, to fund the growth of our business as opposed to paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future.

An active trading market for our common stock may not be sustained. If an active trading market is not sustained, our ability to raise capital in the future may be impaired.

Given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not be sustained, which could put downward pressure on the market price of our common stock and thereby affect your ability to sell shares you purchased. An inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and impair our ability to acquire other companies or technologies by using our shares as consideration.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our existing securityholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale discussed in our Annual Report on Form 10-K for the year ended December 31, 2023 lapse, the trading price of our common stock could decline. As of December 31, 2024, we had 29,865,030 shares of common stock outstanding. All outstanding shares of common stock, other than shares held by our affiliates will be freely tradable, without restriction, in the public market. In addition, shares of common stock that are subject to outstanding options of ours will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements and Rules 144 and 701 under the Securities Act. If these shares are sold, the trading price of our common stock could decline.

Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

As of December 31, 2024, our executive officers, directors and principal stockholders, in the aggregate, beneficially owned approximately 55% of our outstanding shares of common stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

We will have broad discretion in the use of our cash, cash equivalents, and marketable securities and may invest or spend our cash, cash equivalents, and marketable securities in ways with which you do not agree and in ways that may not increase the value of your investment.

We will have broad discretion over the use of our cash, cash equivalents, and marketable securities. You may not agree with our decisions, and our use of our cash, cash equivalents, and marketable securities may not yield any return on your investment. Our failure to apply these resources effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to use our cash resources.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 1C. CYBERSECURITY

Cyber Risk Management and Strategy

Disc Medicine, under the oversight of the audit committee of the board of directors, has implemented and maintains processes to review and manage enterprise risks, including annual assessments of cybersecurity risks, across the Company.

Our cybersecurity risk management program, which is informed by and incorporates elements of recognized industry standards, is designed to identify, assess, and mitigate critical risks from cybersecurity threats. This program includes, but is not limited to, ongoing monitoring for potential critical risks from cybersecurity threats using automated tools. To support our cybersecurity risk management program, we leverage a managed service provider, or MSP, that provides ongoing support for the protection of our information technology infrastructure as well as a virtual Chief Information Security Officer, or vCISO. Our cybersecurity risk management strategy is informed by periodic conversations with, and risk assessments conducted by, our vCISO.

We have an employee security awareness training program, offered upon employee onboarding and on an annual basis, that is designed to raise awareness of cybersecurity threats across functions as well as to encourage consideration of cybersecurity risks across our Company. As part of this employee training program, we periodically conduct phishing simulations designed to raise employee awareness of such risks.

We have also implemented a process to review contractual requirements related to information security obligations included in our agreements with certain third-party vendors and service providers, as appropriate.

We have not identified any cybersecurity incidents or threats that have materially affected us or are reasonably likely to materially affect us, including our business strategy, results of operations or financial condition; however, like other companies in our industry, we and our third-party vendors may, from time to time, experience threats and security incidents relating to our and our third-party vendors' information systems and infrastructure. For more information, please see Item 1A - Risk Factors.

Governance Related to Cybersecurity Risks

Our Head of Information Technology, or IT, under the oversight of our Chief Legal Officer, is responsible for the administration and maintenance of our cybersecurity risk management program, including the day-to-day oversight of the assessment and management of cybersecurity risks. The individual who currently holds the title of Head of IT has more than 20 years of experience in information security and cybersecurity risk management.

Our Head of IT directly reports to, and meets periodically with, our Chief Legal Officer to discuss and review our cybersecurity risk management processes, with input from the Company's MSP and vCISO, as appropriate.

Our board of directors has delegated oversight of the Company's cybersecurity program to the audit committee of the board of directors. As provided in the audit committee charter, the audit committee is responsible for reviewing and discussing the Company's information security and risk management programs, controls, and procedures, including high-level review of the threat landscape facing the Company and the Company's strategy to mitigate cybersecurity risks and potential breaches. Under the audit committee charter, the audit committee is also responsible for reviewing the recovery and communication plans for any unplanned outage, cybersecurity incident, or data breach.

Our Head of IT, twice a year, provides reports to the audit committee on the status of our cybersecurity program, including measures implemented to monitor and address risks from cybersecurity threats, as appropriate. He also reports on a quarterly basis to the executive committee on cybersecurity and information technology matters. The chair of the audit committee provides periodic reports on cybersecurity risk management to the full board of directors. Our Chief Legal Officer, on an annual basis, discusses the results of our enterprise risk assessment processes, including risks related to cybersecurity, with the full board of directors.

ITEM 2. PROPERTIES

Our principal office is located at 321 Arsenal Street, Suite 101, Watertown, MA 02472, where we lease and sublease a total of approximately 16,847 square feet of office space. Our leases expire at various dates between November 2026 and December 2029. We believe that these facilities will be adequate for our near-term needs. If required, we believe that suitable additional or substitute space will be available in the future on commercially reasonable terms to accommodate any such expansion of our operations.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be subject to legal proceedings and claims in the ordinary course of business. We are not currently aware of any such proceedings or claims that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Certain Information Regarding the Trading of Our Common Stock

Our common stock commenced trading on the Nasdaq Global Market under the symbol "GMTX" on February 8, 2021. Prior to this time, there was no public market for our common stock. On December 29, 2022, we completed our previously announced merger transaction with Gemini in accordance with the terms of the Agreement and Plan of Merger, or the Merger Agreement, dated August 9, 2022, pursuant to which Gemstone Merger Sub, Inc., or Merger Sub, merged with and into Disc Medicine Opco, Inc. (f/k/a Disc Medicine, Inc.), or Disc Opco, with Disc Opco continuing as a wholly owned subsidiary of Gemini and the surviving corporation of the merger. On December 29, 2022, in connection with, and prior to the completion of, the merger, Gemini effected a 1-for-10 reverse stock split of its common stock. In connection with the closing of the merger, Gemini also changed its name to Disc Medicine, Inc. On December 30, 2022, following the completion of the merger, our common stock began trading on the Nasdaq Capital Market under the symbol "IRON."

Holders of Record

As of February 21, 2025, there were 19 holders of record of shares of our common stock. The actual number of holders of our common stock is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees.

Dividends

We have never declared or paid cash dividends on our common stock, and we do not expect to pay any cash dividends on our common stock in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors and will depend on various factors, including applicable laws, our results of operations, financial condition, future prospects, then applicable contractual restrictions and any other factors deemed relevant by our board of directors.

Securities Authorized for Issuance Under Equity Compensation Plans

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

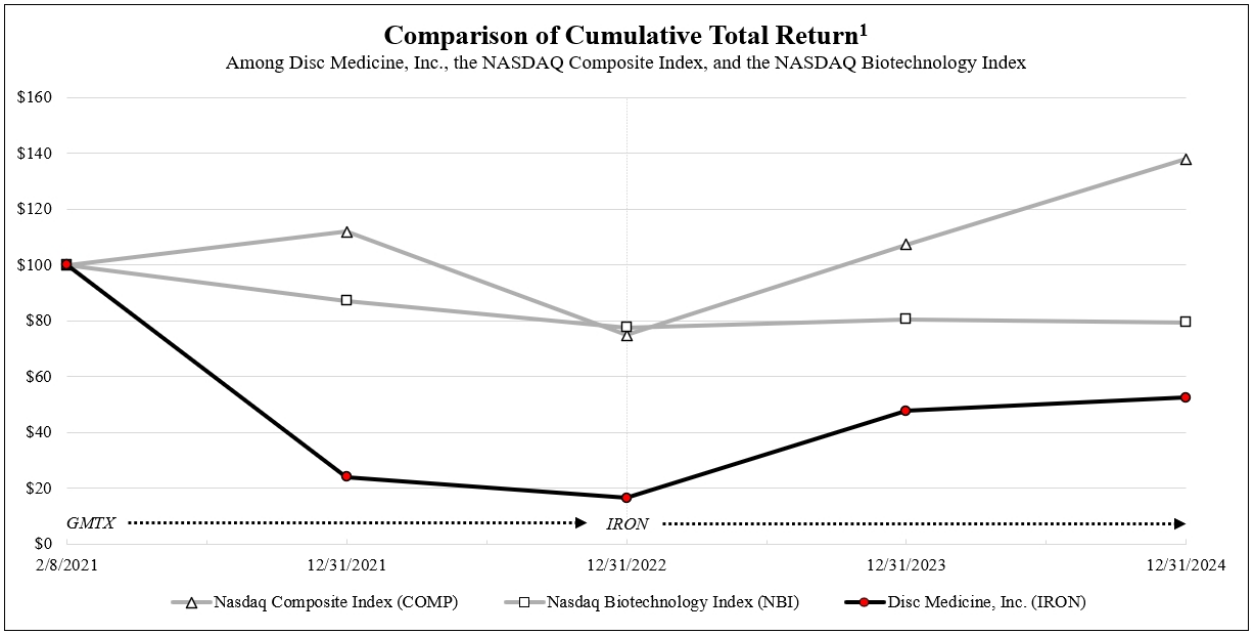
Recent Sales of Unregistered Securities

None.

Stock Performance Graph

The following graph shows a comparison from February 8, 2021 through December 31, 2024 of cumulative total return on assumed investments of $100.00 in cash in each of our common stock, the NASDAQ Composite Index and the NASDAQ Biotechnology Index. Such returns are based on historical results and are not intended to suggest future performance. Data for the NASDAQ Composite Index and the NASDAQ Biotechnology Index assume reinvestment of dividends. Prior to our reverse merger

with Gemini on December 30, 2022, Gemini's stock traded under the symbol "GMTX" on The Nasdaq Global Market and any comparison with Gemini's historical stock prices may not be meaningful.



(1) This performance graph shall not be deemed to be "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, or otherwise subject to the liabilities under that Section, and shall not be deemed incorporated by reference into any filing of Disc Medicine, Inc. under the Securities Act of 1933, as amended.

Use of Proceeds from Initial Public Offering

Not applicable.

Issuer Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

ITEM 6. RESERVED

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing at the end of this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development, and commercialization of novel treatments for patients suffering from serious hematologic diseases. We have assembled a portfolio of clinical and preclinical product candidates that aim to modify fundamental biological pathways associated with the formation and function of red blood cells, specifically heme biosynthesis and iron homeostasis. Our current pipeline includes bitopertin for the treatment of erythropoietic porphyrias, or EPs, including erythropoietic protoporphyria, or EPP, and X-linked protoporphyria, or XLP, and Diamond-Blackfan Anemia, or DBA; DISC-0974 for the treatment of anemia of myelofibrosis, or MF, and anemia of chronic kidney disease, or CKD; and DISC-3405 (formerly MWTX-003) for the treatment of polycythemia vera, or PV, and other hematologic disorders. In addition, our preclinical programs also include DISC-0998 for the treatment of anemia associated with inflammatory diseases. Our approach to product candidate development leverages well-understood molecular mechanisms that have been validated in humans. We believe that each of our product candidates, if approved, has the potential to improve the lives of patients suffering from hematologic diseases.

Bitopertin is the lead product candidate in our heme biosynthesis modulation portfolio. Bitopertin was previously evaluated by F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., or collectively, Roche, in a comprehensive clinical program in over 4,000 individuals in other indications which demonstrated the activity of bitopertin as a glycine transporter 1, or GlyT1, inhibitor and its effect on heme biosynthesis. We are initially developing bitopertin for the treatment of EPs, including EPP and XLP, which are part of a group of severe diseases, known as porphyrias, caused by defects in the heme biosynthesis pathway that cause an accumulation of toxic metabolites referred to as porphyrins, resulting in skin hypersensitivity to sunlight and some types of artificial light. In June and December 2023, we presented interim data from BEACON, a Phase 2 open-label, parallel-dose clinical trial of bitopertin in EPP and XLP patients conducted at sites in Australia. In April 2024, we presented topline data from AURORA, a Phase 2, randomized, double-blind, placebo-controlled clinical trial of bitopertin in EPP patients conducted at sites in the United States. Additional analyses of the BEACON and AURORA trials were presented in June 2024 and in December 2024. In both trials, bitopertin significantly reduced the toxic metabolite, protoporphyrin IX, or PPIX, and was associated with improvements in measures of time spent in sunlight and quality of life, demonstrating a clear association between PPIX reduction and clinical endpoints. In addition, bitopertin was generally well-tolerated. All participants in AURORA and BEACON are eligible to participate in HELIOS, an ongoing open-label, long-term extension study of bitopertin in EPP and XLP. We are also planning APOLLO, a randomized, double-blind, placebo-controlled clinical trial of bitopertin in EPP and XLP patients. In our end-of-Phase 2 meeting, the U.S. Food & Drug Administration, or the FDA, agreed with the potential for reduction of PPIX to serve as a surrogate endpoint to support a potential accelerated approval of bitopertin in EPP and XLP. Under the FDA's Accelerated Approval Program, we would have the potential to submit a New Drug Application, or NDA, for bitopertin in EPP and XLP based on our existing data, and we would be required to conduct a post-marketing confirmatory clinical trial. In our Type C meeting with the FDA in December 2024, we aligned with the FDA on the design of our APOLLO post-marketing confirmatory trial. We plan to initiate the APOLLO trial by mid-2025 and anticipate submitting an NDA for accelerated approval of bitopertin in EPP and XLP in the second half of 2025. We have also entered into a collaborative research and development agreement with the National Institutes of Health, or NIH, to conduct a clinical trial of bitopertin in DBA, which began in July 2023. We are planning additional trials of bitopertin in other indications.

DISC-0974 is the lead product candidate in our iron homeostasis portfolio and was licensed from AbbVie Deutschland GmbH & Co. KG, or AbbVie. DISC-0974 is designed to suppress hepcidin production and increase serum iron levels. We completed a Phase 1 clinical trial in healthy volunteers in the U.S. in June 2022 with results showing an acceptable tolerability profile and evidence of target engagement, iron mobilization and augmented erythropoiesis. We initiated a Phase 1b/2 clinical trial in June 2022 in patients with anemia of MF, and initiated a separate Phase 1b/2 clinical trial in February 2023 in patients with non-dialysis dependent CKD and anemia. We presented interim data from both of these trials in December 2023 as well as additional interim data for anemia of MF in June 2024 and non-dialysis dependent CKD and anemia in October 2024, which additional interim data included safety data and changes in hepcidin, iron, and hemoglobin levels for additional patients, as well as longer follow-up. In December 2024, we presented additional analyses of the Phase 1b study in anemia of MF and initiated the open-label Phase 2 portion of this clinical trial in patients with anemia of MF. We expect to report initial data from this Phase 2 trial in the second half of 2025. We also expect to report data from the multiple-ascending dose, or MAD, portion of the Phase 1b trial in patients with

non-dialysis dependent CKD and anemia in the second half of 2025. We are also planning additional trials of DISC-0974 in other anemias of inflammation. In addition, we are developing a preclinical anti-hemojuvelin, or HJV, monoclonal antibody, DISC-0998, which also targets hepcidin suppression and was licensed from AbbVie. DISC-0998 is designed to increase serum iron levels and has an extended serum half-life as compared to DISC-0974. We believe this profile may be desirable in certain subsets of patients with anemia associated with inflammatory diseases.

Lastly, we are developing DISC-3405, a monoclonal antibody against Transmembrane Serine Protease 6, or TMPRSS6, that we licensed from Mabwell Therapeutics, Inc., or Mabwell. DISC-3405 is part of our iron homeostasis portfolio and is designed to induce hepcidin production and reduce serum iron levels. An IND for DISC-3405 was cleared by the FDA, and a Phase 1 clinical trial in healthy adult volunteers was initiated in October 2023. Interim data was presented from the single-ascending dose, or SAD, portion of the Phase 1 clinical trial of DISC-3405 in healthy volunteers in June 2024. We presented data from the MAD portion of the Phase 1 healthy volunteer study of DISC-3405 in December 2024. We expect to develop DISC-3405 for the treatment of PV and other hematologic disorders, and plan to initiate a Phase 2 clinical trial of DISC-3405 in PV in the first half of 2025.

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $489.9 million. In January 2025 we completed an underwritten offering of shares of our common stock and pre-funded warrants for net proceeds of approximately $243.3 million, after deducting estimated offering expenses payable by us. We believe that our cash, cash equivalents and marketable securities, including the net proceeds of our January 2025 underwritten public offering, will be sufficient to fund our current operating and capital expenditure plans and our debt service obligations into 2028, without taking into account any potential net cash inflows from bitopertin or any other marketed product, if approved during such period. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See *Liquidity and Capital Resources* for additional details.

Financial Operations Overview

Revenue

We have not generated any revenue since our inception and do not expect to generate any revenue from the sale of products in the near future, if at all. If our development efforts are successful and result in commercialization of one or more product candidates or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales, payments from such collaboration or license agreements or a combination thereof.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the research and development of our product candidates. These expenses include:

- employee-related expenses, including salaries, benefits, and stock-based compensation expense, for personnel engaged in research and development functions;
- expenses incurred in connection with our research and development activities, including under agreements with third parties such as consultants, contractors and contract research organizations, or CROs;
- costs related to contract development and manufacturing organizations, or CDMOs, that are primarily engaged to provide drug substance and product for our preclinical studies, clinical trials and research and development programs, as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;
- the costs of acquiring and manufacturing preclinical study and clinical trial materials, including manufacturing registration and validation batches;
- costs related to compliance with quality and regulatory requirements; and
- payments made under third-party licensing agreements.

We expense research and development costs as incurred. Costs incurred for external development activities are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and may be reflected in our consolidated financial statements as prepaid or accrued expenses. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses and expensed as the related goods are delivered or the services are performed or when it is no longer expected that the goods will be delivered or the services rendered.

We typically use our employee and infrastructure resources across product candidates and development programs. We track external development costs by product candidate or development program, but we do not allocate personnel costs or other internal costs to specific product candidates or development programs.

We expect that our research and development expenses will increase substantially as we advance our programs into and through clinical development. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. A change in the outcome of any number of variables with respect to product candidates we may develop could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any product candidates we may develop. The successful development of any product candidate is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to raise additional funds to the extent necessary to complete clinical development of and commercialize our product candidates;
- our ability to establish new licensing or collaboration arrangements and the progress of the development efforts of third parties with whom we may enter into such arrangements;
- our ability to maintain our current research and development programs and to establish new programs;
- the successful initiation, enrollment and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities for any product candidates;
- the availability of raw materials for use in production of our product candidates;
- our ability to establish agreements with third-party manufacturers for supply of product candidate components for our clinical trials;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our other rights in our intellectual property portfolio;
- our ability to commercialize product candidates, if and when approved, whether alone or in collaboration with others; and
- our ability to obtain and maintain third-party insurance coverage and adequate reimbursement for any approved products.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for personnel in executive, finance, commercial, corporate and business development, and administrative functions. Selling, general and administrative expenses also include legal fees relating to patent and corporate matters, including noncapitalizable transaction costs; professional fees for accounting, auditing, tax compliance and administrative consulting services; investor and public relations expenses; commercial planning and market research; director and officer insurance costs and other insurance costs; and facility related expenses including maintenance and allocated expenses for rent and other operating costs.

We anticipate that our selling, general and administrative expenses will increase substantially in the future as we increase our headcount to support our continued research and development and potential commercialization activities including establishing a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval.

Other Income (Expense), Net

Interest Income

Interest income primarily consists of interest earned on cash equivalents, consisting of money market funds, U.S. treasury securities and certificates of deposit, as well as marketable securities, consisting of U.S treasury securities and U.S. government agency securities.

Interest Expense

Interest expense primarily consists of amortization of debt issuance costs and discount and interest expense under the Hercules Loan Agreement.

Results of Operations

Comparison of the Years Ended December 31, 2024 and 2023

The following table summarizes our results of operations for the years ended December 31, 2024 and 2023 (in thousands):

	Year Ended December 31,		
	2024	2023	Change
Operating expenses:			
Research and development	$ 96,671	$ 69,264	$ 27,407
Selling, general and administrative	33,049	21,861	11,188
Total operating expenses	129,720	91,125	38,595
Loss from operations	(129,720)	(91,125)	(38,595)
Other income (expense), net:			
Interest income	21,292	14,797	6,495
Interest expense	(572)	—	(572)
Other expense	(2)	(2)	—
Total other income (expense), net	20,718	14,795	5,923
Loss before income taxes	(109,002)	(76,330)	(32,672)
Income tax expense	(355)	(99)	(256)
Net loss	$ (109,357)	$ (76,429)	$ (32,928)

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2024 and 2023 (in thousands):

	Year Ended December 31,		
	2024	2023	Change
Bitopertin	$ 28,105	$ 17,271	$ 10,834
DISC-0974	16,943	9,136	7,807
DISC-3405	13,934	20,168	(6,234)
Other research programs and expenses	10,150	7,066	3,084
Personnel-related (including equity-based compensation)	27,539	15,623	11,916
Total research and development expenses	$ 96,671	$ 69,264	$ 27,407

Research and development expenses were $96.7 million for the year ended December 31, 2024, compared to $69.3 million for the year ended December 31, 2023. The increase of $27.4 million was primarily due to an $11.9 million increase in personnel-related costs related to higher research and development headcount, including an increase of $5.3 million in stock-based compensation driven by awards granted under our equity compensation plans. Further, external development expenses increased $10.8 million and $7.8 million related to advancing the clinical trials and drug manufacturing activity for bitopertin and DISC-0974, respectively.

These increases were partially offset by a $6.2 million decrease related to the DISC-3405 program which incurred $15.0 million in upfront and milestone license fees in 2023 related to our license agreement with Mabwell which did not recur in 2024. The decrease related to prior year license fees was offset by an increase in external development expense related to increased clinical study and drug manufacturing activity for DISC-3405 in the year ended December 31, 2024.

Selling, General and Administrative Expenses

The following table summarizes our selling, general and administrative expenses for the years ended December 31, 2024 and 2023 (in thousands):

	Year Ended December 31,		
	2024	2023	Change
Personnel-related (including equity-based compensation)	$ 19,367	$ 10,481	$ 8,886
Legal, consulting and professional fees	9,645	7,448	2,197
Other expenses	4,037	3,932	105
Total selling, general and administrative expenses	$ 33,049	$ 21,861	$ 11,188

Selling, general and administrative expenses were $33.0 million for the year ended December 31, 2024, compared to $21.9 million for the year ended December 31, 2023. The increase of $11.2 million was primarily due to an increase of $8.9 million in personnel-related costs due to higher selling, general and administrative headcount, including an increase of $6.0 million in stock-based compensation driven by awards granted under our equity compensation plans.

Other Income (Expense), Net

Other income (expense), net was $20.7 million for the year ended December 31, 2024, compared to $14.8 million for the year ended December 31, 2023. The change of $5.9 million was primarily due to an increase in interest income based on increases in our cash, cash equivalents and marketable securities balances.

Income Tax Expense

Income tax expense was $0.4 million for the year ended December 31, 2024, compared to $0.1 million for the year ended December 31, 2023. The expense relates to state income tax resulting from an increase in interest income.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses. We expect to continue to incur significant expenses and operating losses in the foreseeable future as we advance the clinical development of our product candidates. We expect that our research and development and selling, general and administrative costs will continue to increase significantly, including in connection with conducting clinical trials and manufacturing for our product candidates to support commercialization and providing selling, general and administrative support for our operations, including the costs associated with operating as a public company. As a result, we may need additional capital to fund our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements or other sources. See “Risk Factors” included within Item 1A of this Annual Report on Form 10-K for additional risks associated with our substantial capital requirements.

To date, we have funded our operations primarily with proceeds from the sale of our convertible preferred stock and common stock, the proceeds from the merger with Gemini, proceeds from various private and public sales of our equity securities, and proceeds from borrowings under the Hercules Loan Agreement. Through December 31, 2024, we have received net proceeds of $144.5 million from sales of our Series Seed, Series A and Series B convertible preferred stock, $89.5 million from the merger with Gemini, $477.6 million from various private and public sales of our equity securities, and $27.6 million from borrowings under the Hercules Loan Agreement. As of December 31, 2024, we had cash, cash equivalents, and marketable securities of $489.9 million.

In addition, in January 2025 we completed an underwritten offering of shares of our common stock and pre-funded warrants. The net proceeds from the offering are expected to be approximately $243.3 million, after deducting the underwriting discount and estimated offering expenses. The underwritten offering is described in more detail in *Note 18 - Subsequent Events* to our consolidated financial statements.

We have incurred significant operating losses since inception and, as of December 31, 2024, had an accumulated deficit of $298.0 million. In addition, we expect to continue to incur significant and increasing expenses and operating losses for the foreseeable future. We believe that our cash, cash equivalents and marketable securities, including the net proceeds of our January 2025 underwritten public offering, will be sufficient to fund our current operating and capital expenditure plans and our debt service obligations into 2028, without taking into account any potential net cash inflows from bitopertin or any other marketed product, if approved during such period. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. We may also pursue additional cash resources through public or private equity offerings, collaborations or additional debt financings.

Cash Flows

The following table provides information regarding our cash flows for each period presented (in thousands):

	Year Ended December 31,	
	2024	2023
Net cash (used in) provided by:		
Operating activities	$ (93,926)	$ (73,462)
Investing activities	(292,332)	(89)
Financing activities	218,314	239,379
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (167,944)	$ 165,828

Operating Activities

Our cash flows from operating activities are greatly influenced by our use of cash for operating expenses and working capital requirements to support our business. We have historically experienced negative cash flows from operating activities as we invested in developing our portfolio, drug discovery efforts, conducting clinical trials and manufacturing, and related infrastructure. The cash used in operating activities resulted primarily from our net losses adjusted for non-cash charges and changes in components of operating assets and liabilities, which are primarily the result of increased expenses and timing of vendor payments.

During the year ended December 31, 2024, net cash used in operating activities of $93.9 million was primarily due to our net loss of $109.4 million, offset by changes in operating assets and liabilities of $3.2 million and non-cash expenses of $12.2 million which primarily related to stock-based compensation expense of $16.8 million, offset by amortization and accretion of investment securities of $5.3 million.

During the year ended December 31, 2023, net cash used in operating activities of $73.5 million was primarily due to our net loss of $76.4 million and changes in operating assets and liabilities of $3.0 million, offset by non-cash expenses of $5.9 million primarily related to stock-based compensation expense of $5.5 million.

Investing Activities

During the year ended December 31, 2024, net cash used in investing activities was primarily due to purchases of marketable securities of $386.6 million, partially offset by maturities of marketable securities of $94.8 million.

During the year ended December 31, 2023 net cash used in investing activities was due to purchases of property and equipment.

Financing Activities

During the year ended December 31, 2024, net cash provided by financing activities of $218.3 million consisted primarily of net proceeds of $172.5 million from the June 2024 underwritten offering, net proceeds from the issuance of long-term debt of $27.6 million, aggregate net proceeds of $14.8 million from at-the-market offerings, and proceeds from the exercise of stock options of $3.0 million.

During the year ended December 31, 2023, net cash provided by financing activities of $239.4 million consisted primarily of net proceeds of $147.9 million from sales of common stock and pre-funded warrants in a public follow-on offering, net proceeds of $62.4 million from sales of common stock and pre-funded warrants in a registered direct offering and aggregate net proceeds of $26.4 million from the at-the-market offerings.

Future Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates into and through clinical development and operate as a public company. Our funding requirements and the timing and amount of our operating expenditures will depend largely on:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates or any future product candidates we may develop;
- the costs, timing and outcome of regulatory review of our product candidates;
- changes in laws or regulations applicable to any product candidates we may develop, including but not limited to clinical trial requirements for approvals;
- the cost and timing of obtaining materials to produce adequate product supply for any preclinical or clinical development of any product candidate we may develop;

- the effect of competing technological and market developments;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate we may develop for which we obtain marketing approval;
- the amount and timing of revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; and
- the legal costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that may take longer than we anticipate to become commercially available, if they become commercially available at all. Accordingly, we may need to obtain substantial additional funds to achieve our business objectives.

Until such time, if ever, as we can generate substantial revenue from product sales, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Other Commitments

The following table summarizes our contractual obligations as of December 31, 2024 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods (in thousands):

	Payments Due by Period				
	Total	Less Than 1 Year	1 to 3 Years	3 to 5 Years	More Than 5 Years
Operating lease commitments[1][2]	$ 3,981	$ 821	$ 1,402	$ 1,758	$ —
Total	$ 3,981	$ 821	$ 1,402	$ 1,758	$ —

(1) Amounts reflect contractual rent payments due for our leased and subleased office spaces in Watertown, Massachusetts as of December 31, 2024 and include contractual payments due under our forward-starting lease which is expected to commence in December 2026. The term date for our existing lease is December 31, 2029 and the term date for our existing sublease is November 30, 2026.
(2) Table excludes the impact of tenant improvement allowance reimbursements of $0.8 million which are expected to be received in less than one year. Of this total, $0.5 million relates to our existing leased office space and $0.3 million relates to our forward-starting lease.

We enter into contracts in the normal course of business with CROs, CDMOs and other third parties for preclinical studies, clinical trials and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancelable by us upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation and, in the case of certain arrangements with CROs and CDMOs, may include non-cancelable fees. These payments are not included in the table above as the amount and timing of such payments are not fixed and estimable.

We have also entered into license agreements under which we are obligated to make specified milestone and royalty payments. We have not included future payments under these agreements in the table of contractual obligations above since the payment obligations under these agreements are contingent upon future events such as regulatory milestones or generating product sales. We are unable to estimate the timing or likelihood of achieving these milestones or generating future product sales. For additional

information about our license agreements and amounts that could become payable in the future under such agreements, see our consolidated financial statements.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and costs and expenses in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in *Note 2 - Summary of Significant Accounting Policies* to our consolidated financial statements, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Research and Development Contract Costs and Accruals

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued and prepaid research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and makes adjustments, if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials; and
- CDMOs in connection with the production of preclinical study and clinical trial materials.

The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period.

Stock-Based Compensation Expense

We measure stock-based awards granted to employees, directors, and nonemployees based on their fair value on the date of the grant and recognize compensation expense for those awards over the requisite service period, which is generally the vesting period of the respective award. For stock-based awards with service-based vesting conditions, we recognize compensation expense using the straight-line method. For stock-based awards with performance-based vesting conditions, we use the accelerated attribution method to expense the awards over the implicit service period based on the probability of achieving the performance conditions.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model, which requires inputs based on certain subjective assumptions, including:

- *Fair value of our common stock:* Prior to the merger closing, we determined the estimated fair value of our common-stock based on third-party valuations and certain other relevant objective and subjective factors. After the close of the merger, the fair value of our common stock is determined based on the quoted market price of our common stock. See *Determination of the Fair Value of Common Stock* below for additional details.
- *Expected stock price volatility:* Due to the lack of company-specific historical and implied volatility data, we determine the volatility for awards granted based on an analysis of reported data for a group of guideline companies that issued

options with substantially similar terms. The expected volatility has been determined using a weighted average of the historical volatility measures of this group of guideline companies. We expect to continue to do so until such time that we have adequate historical data regarding the volatility of our own traded stock price.

- *Expected term of the option.* The expected term of our stock options granted to employees has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options, using the average between the vesting date and the contractual term.
- *Risk-free interest rate.* The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award.
- *Expected dividend yield:* We have not paid, and do not anticipate paying, cash dividends on our common stock; therefore, the expected dividend yield is assumed to be zero.

If any assumptions used in the Black-Scholes option pricing model changed significantly, stock-based compensation expense for future awards may differ materially compared with the expense for awards granted previously. We will continue to use judgment in evaluating the assumptions related to our stock-based compensation on a prospective basis. As we continue to accumulate additional data related to our common stock, we may refine our estimates, which could materially impact our future stock-based compensation expense.

Determination of the Fair Value of Common Stock

Prior to the merger closing, the estimated fair value of our common stock had been determined by our board of directors as of the date of each option grant with input from management, considering our most recently available third-party valuation of common stock, and our board of directors' assessment of additional objective and subjective factors that we believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*.

Our common stock valuations were prepared using either an option pricing method, or OPM, or a hybrid method, both of which used market approaches to estimate our enterprise value. The OPM treats common stock and convertible preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the convertible preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock.

The hybrid method is a probability-weighted expected return method, or PWERM, by which the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock.

The assumptions underlying these valuations were highly complex and subjective and represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and stock-based compensation expense could be materially different.

Upon closing of the merger, a public trading market for our common stock has been established and it is no longer necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock is now determined based on the quoted market price of our common stock.

Recently Issued and Adopted Accounting Pronouncements

A description of recently issued and certain recently adopted accounting pronouncements that have or may potentially impact our financial position and results of operations is included in *Note 2 - Summary of Significant Accounting Policies* to our consolidated financial statements.

Emerging Growth Company and Smaller Reporting Company Status

Prior to December 31, 2024, we were an "emerging growth company", as defined in the Jumpstart Our Business Startups Act of 2012, and a "smaller reporting company", as defined under the Exchange Act. As such, we were eligible for exemptions from

various reporting requirements applicable to other public companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 and reduced disclosure obligations regarding executive compensation. As of December 31, 2024, we are no longer an emerging growth company or smaller reporting company. In accordance with SEC rules, we are availing ourselves of the exemptions from disclosure requirements, including certain of the reduced and scaled disclosure obligations, that are available to smaller reporting companies in this Annual Report on Form 10-K. However, beginning with our Quarterly Report on Form 10-Q for the quarter ending March 31, 2025, we will no longer be permitted to take advantage of the reduced reporting requirements applicable to smaller reporting companies.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $489.9 million, which consisted of cash, money market funds, U.S. treasury securities and U.S. government agency securities, which are classified as available-for-sale securities. As of December 31, 2023, we had cash and cash equivalents of $360.4 million, which consisted of cash, money market funds and U.S. treasury securities. Our primary investment objectives are the preservation of capital and the maintenance of liquidity, and our investment policy defines allowable investments based on quality of the institutions and financial instruments designed to minimize risk exposure. Our exposure to interest rate sensitivity is affected by changes in the general level of U.S. interest rates. Our marketable securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term maturities and low risk profiles of our investments, we do not anticipate a significant exposure to interest rate risk. If market interest rates were to increase immediately and uniformly by one percentage point from levels as of December 31, 2024, the net fair value of our marketable securities would decrease by approximately $1.4 million.

Our employees and operations are primarily located in the United States. We have, from time to time, engaged in contracts with contractors or other vendors in a currency other than the U.S. dollar. To date, we have had minimal exposure to fluctuations in foreign currency exchange rates as the time period between the date that transactions are initiated, and the date of payment or receipt of payment is generally of short duration. Accordingly, we believe we do not have a material exposure to foreign currency risk.

Inflation generally affects us by increasing our cost of labor and contract research. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2024 and 2023.

ITEM 8. CONSOLIDATED FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

DISC MEDICINE, INC.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Disc Medicine, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Disc Medicine, Inc. (the Company) as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2024, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated February 27, 2025 expressed an adverse opinion thereon.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Accrued External Research and Development Expenses

Description of the Matter	As of December 31, 2024, the Company had recognized accrued external research and development expenses of approximately $7.1 million. As described in Note 2 to the consolidated financial statements, the Company's determination of accrued external research and development expenses incurred at each reporting period incorporates judgment and utilizes various assumptions as payments for such activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred. Such judgments and assumptions include an evaluation of the information provided to the Company by third parties on actual costs incurred, the time period over which services will be performed and the level of effort to be expended in each period. Auditing the Company's accrued external research and development expenses is especially complex due to the significant volume of transactions and the use of third-party data involved in determining the accrual balance that was accumulated from multiple sources. In addition, in certain circumstances, the determination of the nature and level of services that have been received during the reporting period requires judgment because the timing and pattern of vendor invoicing did not correspond to the level of services provided and invoicing from clinical study sites and other vendors may not yet be available to management.
How We Addressed the Matter in Our Audit	To test the accrued external research and development expenses, our audit procedures included, among others, testing the completeness and accuracy of the underlying data used in the estimate, including, but not limited to, estimated project duration, research and manufacturing services incurred to date and terms of contractual arrangements. To assess the reasonableness of the data, we corroborated the progress of

the underlying research, development and manufacturing service arrangements with Company personnel and obtained direct third-party confirmations supporting the contractual arrangements and estimated costs incurred to date. We recalculated the accrual based on executed contracts with the clinical research organizations, contract development and manufacturing organizations and research institutions. We also tested subsequent invoicing received from third parties to assess the impact to the accrual at the balance sheet date and compared that to the Company's estimates.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2020.

Boston, Massachusetts

February 27, 2025

DISC MEDICINE, INC.
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 192,434	$ 360,382
Marketable securities	297,447	—
Prepaid expenses and other current assets	3,734	5,280
Total current assets	493,615	365,662
Property and equipment, net	751	170
Right-of-use assets, operating leases	1,569	1,930
Other assets	838	234
Total assets	$ 496,773	$ 367,996
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 7,948	$ 12,629
Accrued external research and development expenses	7,141	1,986
Other accrued expenses	8,097	6,159
Operating lease liabilities, current	130	665
Total current liabilities	23,316	21,439
Long-term debt, net	28,322	—
Operating lease liabilities, non-current	1,548	1,436
Total liabilities	53,186	22,875
Commitments and contingencies (Note 15)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized, no shares issued or outstanding as of December 31, 2024 and December 31, 2023	—	—
Common stock, $0.0001 par value; 100,000,000 shares authorized as of December 31, 2024 and December 31, 2023; 29,865,030 and 24,360,233 shares issued and outstanding as of December 31, 2024 and December 31, 2023, respectively	3	2
Additional paid-in capital	741,297	533,764
Accumulated other comprehensive income	289	—
Accumulated deficit	(298,002)	(188,645)
Total stockholders' equity	443,587	345,121
Total liabilities and stockholders' equity	$ 496,773	$ 367,996

The accompanying notes are an integral part of these consolidated financial statements.

DISC MEDICINE, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2024	2023
Operating expenses:		
Research and development	$ 96,671	$ 69,264
Selling, general and administrative	33,049	21,861
Total operating expenses	129,720	91,125
Loss from operations	(129,720)	(91,125)
Other income (expense), net:		
Interest income	21,292	14,797
Interest expense	(572)	—
Other expense	(2)	(2)
Total other income (expense), net	20,718	14,795
Loss before income taxes	(109,002)	(76,330)
Income tax expense	(355)	(99)
Net loss	$ (109,357)	$ (76,429)
Net loss per share, basic and diluted	$ (3.96)	$ (3.42)
Weighted-average common shares outstanding, basic and diluted	27,606,022	22,315,877
Comprehensive loss:		
Net loss	$ (109,357)	$ (76,429)
Other comprehensive income:		
Foreign currency translation adjustments	(27)	—
Unrealized gain on marketable securities	316	—
Total other comprehensive income	289	—
Comprehensive loss	$ (109,068)	$ (76,429)

The accompanying notes are an integral part of these consolidated financial statements.

DISC MEDICINE, INC.
CONSOLIDATED STATEMENTS STOCKHOLDERS' EQUITY
(In thousands, except share and per share amounts)

	Common Stock $0.0001 Par Value		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2022	17,403,315	$ 2	$ 288,814	$ —	$ (112,216)	$ 176,600
Issuance of common stock upon exercise of stock options	254,432	—	2,763	—	—	2,763
Vesting of restricted common stock	1,916	—	—	—	—	—
Stock-based compensation expense	—	—	5,530	—	—	5,530
Sale of common stock in registered direct offering, net of issuance costs of $80	1,488,166	—	34,148	—	—	34,148
Sale of pre-funded warrants in registered direct offering, net of issuance costs of $66	—	—	28,206	—	—	28,206
Sale of common stock in at-the-market offerings, net of issuance costs of $742	967,264	—	26,422	—	—	26,422
Sale of common stock in follow-on public offering, net of issuance costs of $9,272	3,015,919	—	138,508	—	—	138,508
Sale of pre-funded warrants in follow-on public offering, net of issuance costs of $627	—	—	9,373	—	—	9,373
Issuance of common stock upon exercise of warrants	1,229,221	—	—	—	—	—
Net loss	—	—	—	—	(76,429)	(76,429)
Balance at December 31, 2023	24,360,233	$ 2	$ 533,764	$ —	$ (188,645)	$ 345,121
Issuance of common stock upon exercise of stock options	315,266	—	2,976	—	—	— 2,976
Stock-based compensation expense	—	—	16,815	—	—	— 16,815
Sale of common stock in at-the-market offerings, net of issuance costs of $518	234,449	—	14,790	—	—	— 14,790
Sale of common stock in an underwritten offering, net of issuance costs of $5,472	4,944,000	1	172,511	—	—	— 172,512
Issuance of common stock under employee stock purchase plan	11,082	—	441	—	—	— 441
Foreign currency translation adjustments	—	—	—	(27)	—	— (27)
Unrealized gain on marketable securities	—	—	—	316	—	— 316
Net loss	—	—	—	—	(109,357)	— (109,357)
Balance at December 31, 2024	29,865,030	$ 3	$ 741,297	$ 289	$ (298,002)	$ 443,587

The accompanying notes are an integral part of these consolidated financial statements.

DISC MEDICINE, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,	
	2024	2023
Cash flows from operating activities		
Net loss	$ (109,357)	$ (76,429)
Adjustments to reconcile net loss to net cash used in operations:		
Depreciation and amortization	156	100
Stock-based compensation	16,815	5,530
Amortization/accretion of investment securities	(5,304)	—
Non-cash lease expense	433	290
Non-cash interest expense	127	—
Other non-cash items	(27)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	1,571	(1,166)
Accounts payable	(4,681)	(3,741)
Accrued external research and development expenses	5,155	169
Other accrued expenses	1,681	1,839
Operating lease liabilities	(495)	(54)
Net cash used in operating activities	(93,926)	(73,462)
Cash flows from investing activities		
Purchases of property and equipment	(505)	(89)
Purchases of marketable securities	(386,627)	—
Maturities of marketable securities	94,800	—
Net cash used in investing activities	(292,332)	(89)
Cash flows from financing activities		
Proceeds from issuance of long-term debt, net of issuance costs paid	28,988	—
Payment of debt issuance costs	(1,393)	—
Proceeds from sale of common stock in offerings, net of issuance costs paid	172,512	199,078
Proceeds from sale of pre-funded warrants in offerings, net of issuance costs paid	—	37,579
Proceeds from sale of common stock in at-the-market offerings, net of issuance costs paid	14,790	—
Proceeds from stock option exercises	2,976	2,722
Contributions from employee stock purchase plan	441	—
Net cash provided by financing activities	218,314	239,379
Net (decrease) increase in cash, cash equivalents and restricted cash	(167,944)	165,828
Cash, cash equivalents and restricted cash, beginning of period	360,616	194,788
Cash, cash equivalents and restricted cash, end of period	$ 192,672	$ 360,616

The accompanying notes are an integral part of these consolidated financial statements.

DISC MEDICINE, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS (Continued)
(In thousands)

	Year Ended December 31,	
	2024	2023
Supplemental cash flow information		
Cash paid for income taxes	$ 299	$ 112
Cash paid for interest	$ 198	$ —
Cash paid for amounts included in the measurement of operating lease liabilities	$ 718	$ 186
Supplemental disclosure of non-cash activities		
Purchases of property and equipment included in accounts payable and accrued expenses	$ 245	$ 13
Right-of-use assets obtained in exchange for new operating lease liabilities	$ —	$ 1,106
Net increase (decrease) in right-of-use assets related to lease modifications and reassessment events	$ 72	$ (317)
Net increase (decrease) in operating lease liabilities related to lease modifications and reassessment events	$ 72	$ (287)
Deferred issuance costs on sale of pre-funded warrants in registered direct offering included in accounts payable and accrued expenses	$ —	$ 225
Receivable for proceeds from stock option exercises included in other current assets	$ 114	$ 41

The accompanying notes are an integral part of these consolidated financial statements.

1. Organization and Nature of the Business

Disc Medicine, Inc. (together with its subsidiaries, the "Company") is a clinical-stage biopharmaceutical company focused on the discovery, development, and commercialization of novel treatments for patients suffering from serious hematologic diseases. The Company has assembled a portfolio of clinical and preclinical product candidates that aim to modify fundamental biological pathways associated with the formation and function of red blood cells, specifically heme biosynthesis and iron homeostasis. The Company's current pipeline includes bitopertin for the treatment of erythropoietic porphyrias ("EPs") including erythropoietic protoporphyria ("EPP"), X-linked protoporphyria ("XLP"), and Diamond-Blackfan Anemia ("DBA"); DISC-0974 for the treatment of anemia of myelofibrosis ("MF") and anemia of chronic kidney disease ("CKD"); and DISC-3405 for the treatment of polycythemia vera ("PV") and other hematologic disorders. In addition, the Company's preclinical programs include DISC-0998, for the treatment of anemia associated with inflammatory diseases. The Company's approach to product candidate development leverages well-understood molecular mechanisms that have been validated in humans. The Company believes that each of its product candidates, if approved, has the potential to improve the lives of patients suffering from hematologic diseases. The Company was founded in October 2017. The Company's principal offices are located in Watertown, Massachusetts.

The Company is subject to a number of risks and uncertainties common to development stage companies in the biotechnology industry, including, but not limited to, risks associated with completing preclinical studies and clinical trials, receiving regulatory approvals for product candidates, development by competitors of new biopharmaceutical products, dependence on key personnel, reliance on third-party organizations, protection of proprietary technology, compliance with government regulations, the impact of public health crises such as pandemics and the ability to secure additional capital to fund operations. The Company's research and development programs will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Reverse Merger with Gemini

On August 9, 2022, Gemini Therapeutics, Inc., a Delaware corporation ("Gemini"), Gemstone Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Gemini ("Merger Sub"), and Disc Medicine, Inc., a Delaware corporation ("Private Disc"), entered into an Agreement and Plan of Merger and Reorganization (the "Merger Agreement"). The merger was completed on December 29, 2022. In accordance with the Merger Agreement, the Merger Sub merged with and into Private Disc, with Private Disc surviving as a wholly-owned subsidiary of the Company (the "merger"). Gemini changed its name to Disc Medicine Inc., and Private Disc, which remains as a wholly-owned subsidiary of the Company, changed its name to Disc Medicine Opco, Inc. On December 30, 2022, the combined company's common stock began trading on The Nasdaq Capital Market under the ticker symbol "IRON."

Except as otherwise indicated, references herein to "Disc," the "Company," or the "combined company", refer to Disc Medicine, Inc. on a post-merger basis, and the term "Private Disc" refers to the business of privately-held Disc Medicine, Inc., prior to completion of the merger. References to Gemini refer to Gemini Therapeutics, Inc. prior to completion of the merger.

In connection with the merger, each person who was a stockholder of record of Gemini or had the right to receive Gemini's common stock received a contractual contingent value right ("CVR") issued by Gemini subject to and in accordance with the terms and conditions of a Contingent Value Rights Agreement (the "CVR Agreement"), representing the contractual right to receive consideration from the post-closing combined company upon the receipt of certain proceeds from a disposition of Gemini's pre-merger assets (specifically, Gemini's intellectual property assets ("Gemini IP"), which the Company assumed in the merger) during the period that is one year after the closing of the merger, calculated in accordance with the CVR Agreement.

To satisfy its obligations with respect to the CVRs and the Gemini IP, the Company hired an outside firm to attempt to sell the Gemini IP, which firm previously was unsuccessful in finding a buyer for the Gemini IP. In June 2023, the Company received an offer for the Gemini IP and recorded a $1.5 million liability representing the estimated fair value of the Company's obligation to the holders of the CVRs based on the offer. However, the counterparty and the Company were unable to come to an agreement on the terms of the offer.

The disposition period ended December 29, 2023 and no dispositions of Gemini's pre-merger assets were made during the disposition period. Therefore, there were no CVR payments made to the holders and there will not be any future CVR payments to the holders (see *Note 3 - Fair Value Measurements*). As such, the Company's obligations under the CVR Agreement have expired and the CVRs are valueless. In December 2023, the Company recognized the reversal of the $1.5 million liability in the consolidated statements of operations and comprehensive loss. The Company generated no revenue from Gemini's pre-merger assets and incurred approximately $1.6 million in aggregate costs (including maintenance of license agreements, storage, legal and other fees) for the year ended December 31, 2023, to maintain Gemini's pre-merger assets solely in order to fulfill the Company's obligations under the CVR Agreement.

Liquidity and Capital Resources

The Company's consolidated financial statements have been prepared on the basis of the Company continuing as a going concern. The Company expects that its existing cash, cash equivalents and marketable securities as of December 31, 2024 of $489.9 million will enable the Company to fund its planned operating expense and capital expenditure requirements for at least twelve months from the date of issuance of these consolidated financial statements. The Company has incurred recurring losses and negative cash flows from operations since inception. As of December 31, 2024, the Company had an accumulated deficit of $298.0 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future. The future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. There can be no assurance that the Company will ever earn revenues or achieve profitability, or if achieved, that the revenues or profitability will be sustained on a continuing basis. In addition, the Company's preclinical and clinical development activities, manufacturing and commercialization of the Company's product candidates, if approved, may require significant additional financing.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The Company's consolidated financial statements are prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative accounting principles generally accepted in the United States as found in the Accounting Standard Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

Certain reclassifications have been made to prior periods to conform to current period presentation. Reclassification of prior year amounts have been made to separately present accrued external research and development costs from other accrued expenses. There was no impact on total current liabilities or total liabilities resulting from these reclassifications.

Use of Estimates

The preparation of the Company's consolidated financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to accrued research and development expenses; stock-based compensation expense; the fair value of the common stock prior to the effective date of the merger; and income taxes. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it has concluded to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates as there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results may differ materially from those estimates or assumptions.

Segment Information

The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions, resulting in a single reportable segment. The Company has determined that its chief operating decision maker is its Chief Executive Officer. The Company's chief operating decision maker reviews the Company's financial information on a consolidated basis for purposes of allocating resources and assessing financial performance. All of the Company's tangible assets are held in the United States. See *Note 17 - Segment Information* for additional details.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of cash, cash equivalents and marketable securities. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits and limits its exposure to cash risk by placing its cash with high credit quality accredited financial institutions. The Company has concluded that it is not subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company relies, and expects to continue to rely, on a small number of vendors to manufacture supplies and to process its product candidates for its development programs. These programs could be adversely affected by a significant interruption in the manufacturing process or supply chain.

Comprehensive Income (Loss)

Comprehensive income (loss) is the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive income (loss) includes net income (loss) and the change in accumulated other comprehensive income (loss) for the period. Accumulated other comprehensive income (loss) consisted of foreign currency translation adjustments and unrealized gains (losses) on available-for-sale marketable securities during the year ended December 31, 2024.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less at the date of purchase to be cash equivalents. Cash and cash equivalents include cash in readily available checking and money market accounts and U.S. treasury securities with original maturities of three months or less at the date of purchase. Cash equivalents are reflected at fair value based on quoted market prices as further described in *Note 3 - Fair Value Measurements*.

Marketable Securities

The Company classifies its marketable securities as available-for-sale. All of the Company's marketable securities are available to the Company for use in current operations. As a result, the Company classified all of these securities as current assets even though the stated maturity of some individual securities may be one year or more beyond the balance sheet date. Marketable securities are maintained by an investment manager and consist of U.S. treasury and government agency securities. Marketable securities are carried at fair value with the unrealized gains and losses included in accumulated other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense over the life of the underlying marketable security. Although these marketable securities are available to be sold to meet operating needs or otherwise, they are generally held through maturity. The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense), net within the consolidated statements of operations and comprehensive loss. The Company reviews its portfolio of available-for-sale securities, using both quantitative and qualitative factors, to determine if declines in fair value below cost have resulted from a credit loss or other factors. If the decline in fair value is due to credit loss factors, a loss is recognized. To date, the Company has not experienced any credit losses and does not expect it is exposed to any significant credit risk on these investments.

Deferred Transaction Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred transaction costs until such financings are consummated. After consummation of an equity financing, these costs are recorded as a reduction of the proceeds from the transaction, either as a reduction of the carrying value of the preferred stock or in stockholders' equity as a reduction of additional paid-in capital generated as a result of the transaction. Should the in-process equity financing be abandoned, the deferred transaction costs would be expensed immediately as a charge to operating expenses in the consolidated statements of operations and comprehensive loss.

Fair Value Measurements

The Company categorizes its assets and liabilities measured at fair value in accordance with the provisions of ASC 820, *Fair Value Measurements and Disclosures,* which establishes a consistent framework for measuring fair value and expands disclosures for each major asset and liability category measured at fair value on either a recurring or nonrecurring basis. Fair value is defined as the exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

- Level 1—Quoted prices (unadjusted) in active markets for identical assets or liabilities;
- Level 2—Inputs other than quoted prices included within Level 1 that are either directly or indirectly observable; and
- Level 3—Unobservable inputs in which little or no market activity exists, therefore requiring an entity to develop its own assumptions about the assumptions that market participants would use in pricing.

The fair value of the Company's cash equivalents are determined according to the fair value hierarchy described above (see *Note 3 - Fair Value Measurements*). The carrying values of the Company's prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation. Depreciation is calculated using the straight-line method over the estimated useful lives of the assets.

	Estimated Useful Life
Leasehold improvements	Shorter of useful life or remaining life of lease
Computer equipment	3.0 years
Furniture and fixtures	3.0 years

Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated in accordance with the above guidelines once placed into service. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are expensed as incurred.

The Company capitalizes internal costs incurred to develop software for internal use during the application development stage. The Company includes capitalized internally developed software subject to a cloud computing arrangement within other assets. Amortization of capitalized internally developed software costs is recorded in depreciation expense over the estimated useful life of the related asset of 3.0 years.

Impairment of Long-lived Assets

The Company accounts for the impairment and disposition of long-lived assets in accordance with ASC Topic 360, *Property, Plant, and Equipment*. Long-lived assets, such as property and equipment and right-of-use ("ROU") assets, are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset or asset group may not be recoverable. The recoverability of assets or asset groups to be held and used is measured by a comparison of the carrying amount of an asset or asset group to estimated undiscounted future cash flows expected to be generated by the asset or asset group. If the carrying amount of an asset or asset group exceeds its estimated future cash flows, an impairment charge is recognized for the amount by which the carrying amount of the asset or asset group exceeds the estimated fair value of the asset or asset group. There were no impairment losses recorded for the years ended December 31, 2024 and 2023.

Leases

The Company accounts for its leases under ASC Topic 842, *Leases*. Upon adoption, the Company elected to combine lease and non-lease components when calculating minimum lease payments on new leases for all asset classes. The Company has also elected an accounting policy to forgo the recognition of lease assets or liabilities for short-term leases. Short-term leases are defined, in accordance with the standard, as those with terms of one year or less and do not include an option to purchase the underlying asset that the lessee is reasonably certain to exercise.

The Company determines if an arrangement is or contains a lease at contract inception. The Company accounts for a contract as a lease when it has the right to direct the use of the asset for a period of time while obtaining substantially all of the asset’s economic benefits. The Company determines the initial classification and measurement of right-of-use assets and lease liabilities at the lease commencement date and thereafter at the modification date, if modified.

Right-of-use assets represent the Company's right to control the underlying assets under lease, and the lease liability is the Company's obligation to make the lease payments related to the underlying assets under lease, over the contractual term. Right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of future minimum fixed lease payments to be made over the lease term. The Company uses the non-cancellable lease term unless it is reasonably certain that a renewal or termination option will be exercised. When available, the Company will use the rate implicit in the lease to discount lease payments to present value. As most leases do not provide an implicit rate, the Company will estimate the incremental borrowing rate to discount the lease payments. The Company estimates the incremental borrowing rate based on the rates of interest that the Company would have to pay to borrow an amount equal to the lease payments on a collateralized basis, over a similar term, and in a similar economic environment. The right-of-use asset also includes any lease prepayments and initial direct costs, offset by lease incentives.

Certain lease agreements contain variable lease payments which are not included in the measurement of the lease liability. Variable lease payments relate to taxes, insurance, utilities, and common area maintenance ("CAM"). These variable lease payments are recognized in the consolidated statements of operations and comprehensive loss in the period in which the obligation for those payments is incurred.

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including salaries and bonuses, stock-based compensation, employee benefits, facilities costs, depreciation, external costs of vendors engaged to conduct preclinical development activities and clinical trials, manufacturing expenses, as well as the costs of licensing technology.

Nonrefundable prepayments for goods or services that will be used or rendered for future research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed, or when it is no longer expected that the goods will be delivered or the services rendered.

If the Company acquires an asset or group of assets under an in-licensing arrangement that does not meet the definition of a business under ASC Topic 805, *Business Combinations*, and the acquired in-process research and development does not have an alternative future use, any related upfront license payment is expensed as incurred in accordance with guidance in ASC Topic 730, *Research and Development*. Where contingent milestone payments are due to third parties under license or other agreements, the milestone payment obligations are recognized as expense when achievement of the contingent milestone is probable, which is generally upon achievement of the milestone event. Any contingent payments that qualify as a derivative liability are recognized at fair value on the Company's consolidated balance sheets. Annual maintenance fees under license agreements are expensed in the period in which they are incurred. Contingent payments for assets acquired are expensed as incurred or capitalized and amortized based on the nature of the associated asset at the date the payment is recognized. Royalties owed on sales of the products licensed pursuant to license agreements are expensed in the period the related revenues are recognized.

The Company has entered into various research, development and manufacturing contracts with research institutions and other companies primarily in the United States, including contracts with third-party contract research organizations and contract development and manufacturing organizations. These agreements are generally cancelable, and related costs are recorded as research and development expenses as incurred. The Company records accrued liabilities for estimated ongoing research, development and manufacturing costs and prepaid expenses for payments made in advance of work performed. When billing terms under these contracts do not coincide with the timing of when the work is performed, the Company is required to make estimates of outstanding obligations to those third parties as of period end. Any accrual estimates are based on a number of factors, including the Company's knowledge of the progress towards completion of the research, development and manufacturing activities, invoicing to date under the contracts, communication from the research institutions and other companies of any actual costs incurred during the period that have not yet been invoiced and the costs included in the contracts. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results may differ from the estimates made by the Company.

Amortization of Debt Discount and Issuance Costs

Long-term debt is initially recorded as the proceeds received by the Company, net of debt issuance costs and discounts. Debt is subsequently stated at amortized cost. Debt issuance costs and discounts and are amortized to interest expense using the effective interest method, over the term of the debt. Debt issuance costs and discounts related to a recognized debt liability are presented in the consolidated balance sheets as a direct deduction from the carrying amount of that debt liability. For tranches not yet drawn, the related debt issuance costs are deferred and recorded as an asset. In the consolidated statements of cash flows, debt issuance costs paid to lenders are netted against the proceeds from the related long-term debt while debt issuance costs paid to third parties, are presented separately within financing activities.

Patent Costs

The Company expenses all costs as incurred in connection with patent applications, including direct application fees, and the legal and consulting expenses related to making such applications due to the uncertainty about the recovery of the expenditure. These costs are included in selling, general and administrative expenses within the Company's consolidated statements of operations and comprehensive loss.

Stock-Based Compensation

The Company accounts for all stock-based awards granted to employees and non-employees as stock-based compensation expense at fair value in accordance with ASC 718, *Compensation - Stock Compensation*. The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model, which requires inputs based on certain subjective assumptions, including the fair value of the Company's common stock on the date of grant, the expected stock price volatility, the expected term of the option, the risk-free interest rate for a period that approximates the expected term of the option and the Company's expected dividend yield. Due to the lack of company-specific historical and implied volatility data, the Company determines the volatility for awards granted based on an analysis of reported data for a group of guideline companies that issued options with substantially similar terms. The expected volatility has been determined using a weighted-average of the historical

volatility measures of this group of guideline companies. The Company expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options granted to employees has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The Company has not paid, and does not anticipate paying, cash dividends on its common stock; therefore, the expected dividend yield is assumed to be zero. The grant date fair value of restricted stock units and shares of restricted stock granted is based on the fair value of the underlying common stock on the date of grant.

The Company recognizes compensation expense for employees and non-employees over the requisite service period, which is generally the vesting period of the respective award, based on the grant date fair value of the award. For awards that include performance-based vesting conditions expense is recognized using the accelerated attribution method when the performance condition is deemed to be probable. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in the consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified. See *Note 12 - Stock-Based Compensation* for a summary of the stock-based award activity under the Company's stock-based compensation plans.

Determination of Fair Value of Common Stock on Grant Dates

Prior to the merger, due to the absence of an active market for Private Disc's common stock, Private Disc and its Board were required to determine the fair value of Private Disc's common stock at the time of each grant of a stock-based award. Private Disc estimated the fair value of its common stock utilizing methodologies in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*. In determining the exercise prices for options granted, Private Disc considered the estimated fair value of the common stock as of the measurement date. The estimated fair value of the common stock has been determined at each grant date based upon a variety of factors, including prices paid for Private Disc's convertible preferred stock and the rights, preferences, and privileges of Private Disc's Preferred Stock and common stock; Private Disc's stage of development and status of technological developments within Private Disc's research; the illiquid nature of securities in a private company; the prospects of a liquidity event; and the current business climate in the marketplace. Significant changes to the key assumptions underlying the factors used could result in different fair values of common stock at each valuation date.

Private Disc's common stock valuations were prepared using either an option pricing method ("OPM"), or a hybrid method, both of which used market approaches to estimate our enterprise value. The OPM treats common stock and convertible preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeds the value of the convertible preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock.

The hybrid method is a probability-weighted expected return method ("PWERM"), by which the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. In addition to a scenario using the OPM, the hybrid method also considers an initial public offering scenario in which the shares of convertible preferred stock are assumed to convert to common stock. The future value of the common stock in the initial public offering scenario was discounted back to the valuation date at an appropriate risk adjusted discount rate. In the hybrid method, the present value indicated for each scenario was probability weighted to arrive at an indication of value for the Private Disc's common stock. Private Disc utilized significant estimates and assumptions in determining the fair value of its equity and equity-based awards.

Substantially all of the awards granted by the Company are either new hire grants or routine annual grants. Management evaluates its award grants and modifications contemporaneously and if any are determined to be spring-loaded, the Company will adjust the fair value.

Income Taxes

Income taxes have been accounted for using the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax

assets and liabilities are measured using enacted tax rates applicable to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the period that includes the enactment date. A valuation allowance against deferred tax assets is recorded if, based upon the weight of all available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. Since the Company has generated operating losses and expects to continue to incur future losses, the net deferred tax assets have been fully offset by a valuation allowance.

The Company accounts for income taxes in accordance with authoritative accounting guidance which states the impact of an uncertain income tax position is recognized at the largest amount that is "more likely than not" to be sustained upon audit by the relevant taxing authority. There are no unrecognized tax benefits included in the Company's consolidated balance sheets at December 31, 2024 or 2023. The Company's practice is to recognize interest and penalties related to income tax matters in income tax expense. The Company has not recognized interest or penalties related to income tax matters in its consolidated statements of operations and comprehensive loss since inception.

The Company files federal income tax returns in the United States, the Netherlands and Australia and state income tax returns in Massachusetts and various other state jurisdictions. The Company's income tax returns are subject to review and tax assessment from an income tax examination. As of December 31, 2024, the Company was not under examination by the Internal Revenue Service or other jurisdictions for any tax year.

Net Loss Per Share

Basic and diluted net loss per share is computed by dividing net loss by the weighted-average common shares outstanding. The weighted-average common shares outstanding used in the basic and diluted net loss per share calculation includes the pre-funded warrants issued in connection with the Company's follow-on public offering in June 2023 and registered direct offering in February 2023 as the pre-funded warrants are exercisable for nominal cash consideration. In periods in which the Company reports a net loss, diluted net loss per share is generally the same as basic net loss per share since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Recently Adopted Accounting Pronouncements

On January 1, 2024, the Company adopted ASU 2023-07, *Segment Reporting: Improvements to Reportable Segment Disclosures*, which is intended to improve reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses. The guidance is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Refer to *Note 17 - Segment Information* for additional details.

Recently Issued Accounting Pronouncements Not Yet Adopted

Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on the Company's consolidated financial statements upon adoption.

3. Fair Value Measurements

The following tables present information about the Company's assets and liabilities that are regularly measured and carried at fair value on a recurring basis and indicate the level within the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value, which is described further within *Note 2 - Summary of Significant Accounting Policies*.

Financial assets and liabilities measured at fair value on a recurring basis are summarized as follows (in thousands):

	December 31, 2024		
	Level 1	Level 2	Level 3
Assets			
Cash equivalents:			
Money market funds	$ 88,735	$ —	$ —
U.S. treasury securities	—	91,916	—
Total cash equivalents	88,735	91,916	—
Marketable securities:			
U.S. treasury securities	—	189,663	—
U.S. government agency securities	—	107,784	—
Total marketable securities	—	297,447	—
Total assets	$ 88,735	$ 389,363	$ —

	December 31, 2023		
	Level 1	Level 2	Level 3
Assets			
Cash equivalents:			
Money market funds	$ 55,001	$ —	$ —
U.S. treasury securities	—	255,419	—
Total cash equivalents	55,001	255,419	—
Total assets	$ 55,001	$ 255,419	$ —

The fair value of the Company's Level 1 cash equivalents, consisting of money market funds, is based on quoted market prices in active markets with no valuation adjustment. The fair value of the Company's Level 2 cash equivalents and marketable securities, consisting of U.S. treasury and U.S. government agency securities with original maturities of three months or less and twelve months or less, respectively, are determined through third-party pricing services. The amortized cost of cash equivalents approximates the fair value. There have been no impairments of the Company's assets measured and carried at fair value during the years ended December 31, 2024 and 2023. In addition, there were no changes in valuation techniques or transfers between Level 1, Level 2 and Level 3 financial assets during the years ended December 31, 2024 and 2023. The Company did not have any non-recurring fair value measurements on any assets or liabilities during the years ended December 31, 2024 and 2023.

As described in *Note 1 - Organization and Nature of the Business*, in connection with the merger, the stockholders of Gemini received a CVR to receive consideration from the Company upon its receipt of certain proceeds, resulting from a disposition of Gemini's pre-merger assets within one year after the closing of the merger, calculated in accordance with the CVR Agreement. The disposition period ended December 29, 2023 and no dispositions of Gemini's pre-merger assets were made during the disposition period. Therefore, there were no CVR payments made to the holders and there will not be any future CVR payments to the holders. The fair value of the CVR liability was zero as of December 31, 2024 and 2023, respectively.

4. Marketable Securities

Marketable securities as of December 31, 2024 consisted of the following (in thousands):

		Gross Unrealized		
	Amortized Cost	Gains	Losses	Estimated Fair Value
Marketable securities available-for-sale:				
U.S. treasury securities	$ 189,409	$ 288	$ (34)	$ 189,663
U.S. government agency securities	107,722	85	(23)	107,784
Total available-for-sale securities	$ 297,131	$ 373	$ (57)	$ 297,447

Marketable securities as of December 31, 2023 were zero. As of December 31, 2024, all marketable securities had an original maturity date of twelve months or less. The aggregate fair value of marketable securities with unrealized losses was $51.0 million as of December 31, 2024 and zero as of December 31, 2023. As of December 31, 2024 and December 31, 2023, five investments and no investments, respectively, were in an unrealized loss position. All such investments have been in an unrealized loss position for less than a year and these losses are considered temporary. The Company has the ability and intent to hold these investments until a recovery of their amortized cost, which may not occur until maturity. The Company expects that U.S. treasury and U.S. government agency securities are subject to minimal credit risk. As a result, the Company did not record any charges for credit-related impairments for its available-for-sale securities for the years ended December 31, 2024 and 2023.

5. Cash, Cash Equivalents and Restricted Cash

Cash, cash equivalents and restricted cash consisted of the following (in thousands):

	December 31, 2024	December 31, 2023
Cash and cash equivalents	$ 192,434	$ 360,382
Restricted cash	238	234
Total cash, cash equivalents and restricted cash as shown on the consolidated statements of cash flows	$ 192,672	$ 360,616

The Company's restricted cash balance, which is classified within other assets on the consolidated balance sheets, consists of letters of credit related to leased and subleased office space in Watertown, Massachusetts. The Company is required to maintain a separate cash balance to secure its letters of credit.

6. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	December 31, 2024	December 31, 2023
Leasehold improvements	$ 566	$ —
Furniture and fixtures	355	184
Computer equipment	231	231
Less: Accumulated depreciation	(401)	(245)
Property and equipment, net	$ 751	$ 170

Depreciation expense for the years ended December 31, 2024 and 2023 was $0.2 million and $0.1 million, respectively.

7. Other Accrued Expenses

Other accrued expenses consisted of the following (in thousands):

	December 31, 2024	December 31, 2023
Accrued employee-related expenses	$ 7,211	$ 5,790
Accrued professional fees	531	317
Accrued other	355	52
Total other accrued expenses	$ 8,097	$ 6,159

8. Leases

The Company has an operating lease and sublease for office space which serves as the Company's corporate headquarters. The Company's sublease and lease expire in November 2026 and December 2029, respectively. The Company's leases do not contain any material residual value guarantees or restrictive covenants. Operating leases are recognized on the consolidated balance sheets as right-of-use assets, operating leases, operating lease liabilities, current and operating lease liabilities, non-current. Operating lease expense is recognized on a straight-line basis over the lease term within the Company's consolidated statements of operations and comprehensive loss.

In August 2024, the Company amended its lease of 7,566 square feet of office space which serves as the Company's corporate headquarters. The lease was amended to extend the term from November 30, 2026 through December 31, 2029. The Company accounted for this amendment as a modification which resulted in the application of a new incremental borrowing rate and the recognition of an additional right-of-use asset and lease liability of $0.1 million, inclusive of a tenant improvement allowance of $0.5 million.

In connection with the August 2024 amendment, the Company entered into a forward-starting lease of office space on the second floor of its corporate headquarters, which the Company is currently subleasing. The forward-starting lease is expected to commence on December 1, 2026 immediately following the conclusion of the sublease, and has a term date of December 31, 2029. The Company expects to make undiscounted payments of $1.0 million over the term of the lease, inclusive of a tenant improvement allowance of $0.3 million. The impact of the forward-starting lease is not currently recorded on the Company's consolidated balance sheets as of the reporting date. The Company will record the impact upon lease commencement.

The components of lease expense were as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Operating lease costs	$ 641	$ 422
Variable lease costs	486	159
Total lease expense	$ 1,127	$ 581

Other information related to the Company's leases is as follows:

	Year Ended December 31,	
	2024	2023
Weighted-average remaining lease term	3.5 years	2.9 years
Weighted-average discount rate	10.3%	10.0%

The following table presents the future minimum lease payments under the Company's lease liabilities as of December 31, 2024, (in thousands):

	Operating Leases
2025	$ 324
2026	768
2027	285
2028	389
2029	401
Total minimum lease payments	$ 2,167
Less: imputed interest	(489)
Lease liabilities	$ 1,678

Operating lease maturity amounts in the table above include tenant improvement allowance reimbursements of $0.5 million which are expected to be received in 2025. The table does not include contractual rent payments under the forward-starting lease which are expected to total $1.0 million, net of a tenant improvement allowance of $0.3 million on an undiscounted basis.

9. Long-Term Debt

On November 6, 2024 (the "Closing Date"), the Company entered into a Loan and Security Agreement (the “Hercules Loan Agreement”), with the lenders party thereto (the “Lenders”) and Hercules Capital, Inc., as administrative agent and collateral agent (the “Agent”). The Hercules Loan Agreement provides for up to $200.0 million of senior secured term loans available to the Company in multiple tranches (the “Term Loan Facility”). Under the Hercules Loan Agreement, the Company borrowed an initial amount of $30.0 million on the Closing Date, and at the Company's sole option, can draw an additional $80.0 million on or prior to December 15, 2026, as well as additional term loan advances in an aggregate principal amount of up to $65.0 million during the term of the Term Loan Facility subject to achievement of specified performance milestones, and one additional term loan advance up to an aggregate principal amount of $25.0 million subject to certain terms and conditions. The Company intends to use the proceeds of the Term Loan Facility for working capital and general corporate purposes.

The Term Loan Facility will mature on December 1, 2029 (the “Maturity Date”). The outstanding principal balance of the Term Loan Facility bears cash interest at a floating annual rate equal to the greater of (i) 8.25% and (ii) the sum of the Prime Rate and 1.75%. Accrued interest is payable monthly following the funding of each term loan advance. Borrowings under the Hercules Loan Agreement are repayable in monthly interest-only payments through November 2028. At the end of the interest-only payment period, borrowings under the Hercules Loan Agreement are repayable in equal monthly payments of principal and accrued interest until the Maturity Date. At the Company's option, the Company may prepay all or a portion of the outstanding borrowings, subject to a prepayment fee of 3.0% of the principal amount if prepayment occurs during the 18 months following the Closing Date, 2.0% after 18 months following the Closing Date but prior to 36 months following the Closing Date, and 1.0% thereafter.

The obligations under the Hercules Loan Agreement are secured, subject to customary permitted liens and other agreed-upon exceptions, by a first-priority perfected security interest in all of the tangible and intangible assets of the Company, other than intellectual property. The Hercules Loan Agreement includes customary repayment and prepayment terms, events of default, affirmative and negative covenants and representations and warranties. Additionally, the Hercules Loan Agreement contains a minimum cash covenant that requires the Company to maintain certain levels of cash in accounts subject to a control agreement in favor of the Agent at all times beginning on the first day on or after January 1, 2027 (or January 1, 2028, if the Company achieves a certain milestone) on which the aggregate principal amount of the term loan advances is then more than $50.0 million; provided, however, the minimum cash covenant will be waived during all times that the Company's market capitalization is greater than or equal to $1.0 billion. The Hercules Loan Agreement also includes a subjective acceleration clause for circumstances which could be reasonably expected to have a material adverse effect on the Company.

The Company recorded debt discount and debt issuance costs of $1.8 million upon closing of the Hercules Loan Agreement. The Hercules Loan Agreement also provides for a final payment ("End of Term Charge"), payable upon maturity or the repayment of the obligations in full or in part (on a pro rata basis), equal to 6.75% of the aggregate principal amount of term loans advanced to the Company and repaid on such date, which is being accrued on the Company's consolidated balance sheets. As of December 31, 2024, the amount accrued for the End of Term Charge was $0.1 million.

Debt discount and unamortized debt issuance costs were recorded as a reduction of the carrying amount on the term loan and are amortized as interest expense using the effective-interest method. In addition, unamortized deferred financing costs of $0.6 million were recorded in other assets as of December 31, 2024 related to the Company's right to borrow additional amounts in the future. Interest expense for the year ended December 31, 2024 was $0.6 million.

As of December 31, 2024, the carrying value of the Term Loan Facility approximates its fair value.

The obligations under the Term Loan Facility as of December 31, 2024 consisted of the following (in thousands):

	December 31, 2024
Principal term loan balance	$ 30,000
Unamortized debt discount and issuance costs	(1,746)
Accrued end of term fee	68
Long-term debt, net	$ 28,322

The annual principal payments due under the Term Loan Facility as of December 31, 2024 were as follows (in thousands):

2025	$ —
2026	—
2027	—
2028	2,200
2029	27,800
Total	$ 30,000

The table of future principal payments excludes the End of Term Charge of $2.0 million, which is due upon the maturity of the loan.

10. Development and License Agreements

License Agreement with Oak Bay Biosciences, Inc. ("OBB")

In December 2024, the Company entered into an out-license agreement with OBB pursuant to which the Company granted an exclusive license, with the right to grant sublicenses, to certain Gemini IP. Under the terms of the agreement, the Company received an upfront payment of $0.2 million with additional consideration receivable upon the achievement of certain clinical, regulatory and sales milestones up to $7.0 million, $35.0 million, and $160.0 million, respectively. As of December 31, 2024, none of the milestones had been achieved and are not considered probable of achievement.

License and Stock Purchase Agreement with AbbVie Deutschland GmbH & Co. KG ("AbbVie")

In September 2019, the Company entered into an agreement with AbbVie, pursuant to which AbbVie granted the Company an exclusive license, with the right to grant sublicenses, to certain AbbVie intellectual property.

Under this agreement, the Company paid a non-refundable, non-creditable upfront fee of $0.6 million. The Company is also obligated to make future payments upon the achievement of certain development, commercialization and sales-based milestones up to $18.0 million, $45.0 million and $87.5 million, respectively on a licensed product-by-licensed product basis. In addition, the Company is also obligated to pay royalties based on net sales of the licensed products on a licensed product-by-licensed product and country-by-country basis. As of December 31, 2024, none of the milestones had been achieved.

The Company's royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the expiration of the last-to-expire valid claim under the licensed intellectual property rights in such country. Unless terminated earlier, the agreement expires upon the expiration of the Company's royalty obligation for all licensed products. AbbVie can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event of a material breach by the Company and failure to cure such breach within a certain period of time. The Company made the first milestone payment associated with the license agreement with AbbVie in January 2025, refer to *Note 18 – Subsequent Events* for additional details.

License Agreement with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. (collectively "Roche")

In connection with a license agreement with Roche, (the "Roche Agreement"), the Company paid Roche an upfront, non-refundable exclusivity payment of $0.5 million in March 2021. Upon execution of the Roche Agreement in May 2021, the Company paid Roche an additional upfront, non-refundable payment of $4.0 million.

The Company is obligated to make contingent payments to Roche up to an aggregate of $50.0 million in development and regulatory milestone payments for development and approval in a first indication and up to an aggregate of $35.0 million in development and regulatory milestone payments for development and approval in a second indication. The Company is also obligated to make contingent payments to Roche up to an aggregate of $120.0 million based on achievement of certain thresholds for annual net sales of licensed products. Roche is also eligible to receive tiered royalties on net sales of commercialized products, at rates ranging from high single-digits to high teens. Pursuant to the terms of the Roche Agreement and in connection with the reverse merger with Gemini, the Company issued 482,313 shares of the Company to Roche for no consideration.

The next potential milestone payments include $10.0 million due upon the initiation of the first Phase 3 clinical trial with a licensed product in a first indication and $15.0 million due upon regulatory approval in the United States with a licensed product in a first indication. As of December 31, 2024, none of the milestones had been achieved.

License Agreement with Mabwell Therapeutics, Inc. ("Mabwell")

In January 2023, the Company entered into an exclusive license agreement with Mabwell, pursuant to which Mabwell granted the Company an exclusive and sublicensable license to certain Mabwell intellectual property.

In connection with the agreement, the Company paid Mabwell an upfront payment of $10.0 million in March 2023. In October 2023, the Company dosed the first patient in the Phase 1 clinical trial for DISC-3405, resulting in a milestone payment of $5.0 million to Mabwell. The next potential milestone payments include $10.0 million due upon the initiation of a Phase 2 clinical trial for DISC-3405 and $5.0 million due upon the initiation of a Phase 1b clinical trial with a licensed product in a second indication. As of December 31, 2024, neither of these milestones had been achieved.

In addition, the Company is obligated to pay certain development and regulatory milestone payments for the licensed products, for up to three indications, up to a maximum aggregate amount of $127.5 million, as well as certain commercial milestone payments for certain licensed product net sales achievements, up to a maximum aggregate amount of $275.0 million. The Company is further obligated to pay a tiered percentage of revenue that the Company receives from its sublicensees ranging from a low third decile percentage to a low first decile percentage. In addition, the Company is obligated to pay Mabwell a royalty on annual net sales of all licensed products at a tiered rate ranging from low single-digits to high single-digits.

During the years ended December 31, 2024 and 2023, the Company recorded research and development expense related to its arrangement with Mabwell of less than $0.1 million and $15.0 million, respectively.

11. Stockholders' Equity

Preferred Stock

The Company was authorized to issue up to 10,000,000 shares of preferred stock with a par value of $0.0001 per share. As of December 31, 2024 no shares or preferred stock were issued or outstanding.

Common Stock

The Company was authorized to issue up to 100,000,000 shares of common stock with a par value of $0.0001 per share. As of December 31, 2024, 29,865,030 shares of common stock were issued and outstanding.

ATM Programs

Cantor Fitzgerald & Co. (“Cantor”)

On November 15, 2024, the Company entered into a Controlled Equity Offering Sales Agreement (the “Cantor ATM Agreement”) with Cantor, pursuant to which the Company may, from time to time in its sole discretion, issue and sell to or through Cantor, acting as sales agent, shares of the Company’s common stock, par value $0.0001 per share having an aggregate offering price of up to $200.0 million. This offering was made pursuant to the Company's effective registration statement on Form S-3 filed in August 2024 (the "August 2024 Shelf"). As of December 31, 2024, there been had no issuances or sales under the Cantor ATM Agreement.

Jefferies LLC

In October 2023, the Company entered into an Open Market Sale Agreement with Jefferies LLC as sales agent (the “ATM Agreement”) to provide for the offering, issuance and sale by the Company of up to $59.7 million of the Company’s common stock from time to time in ATM offerings under the January 2023 Shelf. In November 2023, the Company filed a shelf registration statement on Form S-3 with the SEC, which covered the offering, issuance and sale by the Company of up to an aggregate of $400.0 million of the Company’s common stock, preferred stock, debt securities, warrants or units (the “November 2023 Shelf”). On December 5, 2023, the Company and Jefferies LLC entered into an amendment to the ATM Agreement to increase the aggregate offering price of the shares of common stock that the Company may offer under the ATM Agreement from $59.7 million to $200.0 million. The material terms and conditions of the ATM Agreement otherwise remained unchanged. On November 15, 2024, the Company terminated the ATM Agreement. In connection with the termination, the Company recognized the remaining capitalized issuance costs of $0.6 million as selling, general and administrative expense in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2024.

During the years ended December 31, 2024 and 2023, the Company had sold an aggregate of 234,449 and 141,914 shares of common stock in ATM offerings under the November 2023 Shelf and pursuant to the ATM Agreement, respectively. During the

years ended December 31, 2024 and 2023, aggregate gross proceeds from the transactions were $15.3 million and $7.2 million, respectively, and the Company received $14.8 million and $7.0 million in net proceeds, after deducting placement agent fees and offering expenses, respectively. In total, the Company sold an aggregate of 376,363 shares of common stock in ATM offerings under the November 2023 Shelf and pursuant to the ATM Agreement for aggregate gross proceeds of $22.5 million and aggregate net proceeds received of $21.8 million, after deducting placement agent fees and offering expenses.

SVB Securities LLC

In January 2023, the Company filed a shelf registration statement on Form S-3 with the SEC, which covered the offering, issuance and sale by the Company of up to an aggregate of $300.0 million of the Company's common stock, preferred stock, debt securities, warrants or units (the "January 2023 Shelf"). Subsequently in January 2023, the Company entered into a Sales Agreement (the "Sales Agreement") with SVB Securities LLC, as sales agent, to provide for the offering, issuance and sale by the Company of up to $100.0 million of the Company's common stock from time to time in "at-the-market" ("ATM") offerings under the January 2023 Shelf. Effective June 12, 2023, the ATM program with SVB Securities LLC was suspended. Following the date of the ATM suspension, the Company did not make any further sales of its common stock pursuant to the Sales Agreement. The Sales Agreement was terminated effective September 28, 2023. In connection with the ATM suspension, the Company recognized the remaining capitalized issuance costs of $0.3 million as selling, general and administrative expense in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2023.

During the year ended December 31, 2023, the Company had sold an aggregate of 825,350 shares of common stock in ATM offerings under the January 2023 Shelf and pursuant to the Sales Agreement. During the year ended December 31, 2023, aggregate gross proceeds from the transactions were $20.0 million and the Company received $19.5 million in net proceeds, after deducting placement agent fees and offering expenses.

Underwritten Offering

In June 2024, the Company entered into an underwriting agreement with Leerink Partners LLC related to an underwritten offering of 4,944,000 shares of common stock of the Company, par value $0.0001 per share, at a price to the public of $36.00 per share. The Company received net proceeds of $172.5 million, after deducting the underwriting discount and offering expenses of $5.5 million.

Follow-On Public Offering

In June 2023, the Company issued 3,015,919 shares of its common stock upon the completion of its public follow-on offering, which included the exercise in full by the underwriters of their option to purchase up to 420,000 additional shares of common stock, at a public offering price of $49.00 per share. The Company also sold, in lieu of shares of the Company's common stock, pre-funded warrants to purchase an aggregate of 204,081 shares of common stock at a price of $48.9999 per pre-funded warrant. The Company received aggregate net proceeds of $147.9 million, after deducting offering expenses of $9.9 million. As of December 31, 2024, none of the 204,081 pre-funded warrants had been exercised.

The pre-funded warrants provide that the holder will not have the right to exercise any portion of its warrants if such holder, together with its affiliates, would beneficially own in excess of 24.99% of the number of shares of Common Stock outstanding immediately after giving effect to the exercise (the "Ownership Limit"). Purchasers of the pre-funded warrants may also elect to set the initial Ownership Limit at 4.99%, 9.99% or 19.99%. Upon at least 61 days' prior notice from the holder to the Company, the holder may increase or decrease the Ownership Limit up to 24.99%, provided however that purchasers that select an Ownership Limit of 19.99% or less will only be allowed to increase the Ownership Limit above 19.99% if such increase would not result in a change of control under the rules and regulations of the Nasdaq Stock Market LLC. The pre-funded warrants meet the condition for equity classification and were therefore recorded as a component of stockholders' equity within additional paid-in capital.

Registered Direct Offering

In February 2023, the Company entered into a securities purchase agreement, with certain investors. Pursuant to the securities purchase agreement, the Company sold an aggregate of 1,488,166 shares of the Company's common stock, at a purchase price of $23.00 per share, and with respect to a certain investor, in lieu of shares of the Company's common stock, pre-funded warrants to purchase an aggregate of 1,229,224 shares of the Company's common stock, at a purchase price of $22.9999 per pre-funded warrant, for aggregate net proceeds of $62.4 million, after deducting offering expenses of $0.1 million. In September 2023, all of the pre-funded warrants were exercised in a cashless transaction which resulted in 1,229,221 shares of common stock being issued to the investor.

The pre-funded warrants provide that the holder would not have the right to exercise any portion of its warrants if such holder, together with its affiliates, would beneficially own in excess of 9.99% of the number of shares of the Company's common stock outstanding immediately after giving effect to such exercise (the "Beneficial Ownership Limitation"); provided, however, that the holder may increase or decrease the Beneficial Ownership Limitation by giving 61 days' notice, but not to any percentage in excess

of 19.99%. The investors or their affiliates were beneficial holders of more than 5% of the Company's capital stock. The pre-funded warrants meet the condition for equity classification and were therefore recorded as a component of stockholders' equity within additional paid-in capital at the time of their issuance.

Registration Statements Resulting from the Merger

In January 2023, as a result of the merger, the Company filed a resale registration statement on Form S-3 with the Securities and Exchange Commission ("SEC"), which covered the proposed resale or other disposition by certain stockholders of up to an aggregate of 12,635,956 shares of the Company's common stock. The Company also filed a registration statement on Form S-8 with the SEC, which registered 1,672,599 shares of common stock issuable with respect to Private Disc options assumed by the Company pursuant to the Merger Agreement as well as 2,035,103 additional shares of common stock reserved and available for future issuance under the 2021 Plan and 180,894 additional shares of common stock reserved and available for future issuance under the 2021 Employee Stock Purchase Plan (the "2021 ESPP").

12. Stock-Based Compensation

2017 Stock Option and Grant Plan (the "Private Disc Plan")

Private Disc adopted the Private Disc Plan in November 2017 reserving shares of common stock for issuance to employees, directors, and consultants. The Private Disc Plan allowed for the grant of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards and other stock awards. Recipients of stock options or stock appreciation rights were eligible to purchase shares of Private Disc's common stock at an exercise price equal to the estimated fair market value of such stock on the date of grant. The exercise price could have been less than fair market value if the stock award was granted pursuant to an assumption or substitution for another stock award in the event of a merger or sale of Private Disc. The maximum term of options granted under the Private Disc Plan was ten years, and stock options typically vested over a four-year period. The Board could have assigned vesting terms to the stock options grants as deemed appropriate. Private Disc also had the right of first refusal to purchase any proposed disposition of shares issued under the Private Disc Plan. As it relates to restricted stock awards, Private Disc had the option to repurchase any unvested shares at the original purchase price upon any voluntary or involuntary termination. At the discretion of the Board, unvested shares held by employees, directors and consultants could have accelerated vesting in the event of a change of control of Private Disc unless assumed or substituted by the acquirer or surviving entity. Upon completion of the merger in December 2022, the Company ceased granting awards under the Private Disc Plan.

2021 Stock Option and Incentive Plan

In February 2021, Gemini adopted the 2021 Stock Option and Incentive Plan (the "2021 Plan") reserving shares of common stock to grant incentive stock options or nonqualified stock options for the purchase of common stock, stock appreciation rights, restricted stock awards, restricted stock units, unrestricted stock awards, cash-based awards and dividend equivalent rights to employees, officers, directors and consultants. Upon approving the 2021 Plan in February 2021, Gemini ceased granting awards under its then existing 2017 Stock Option and Grant Plan (the "2017 Gemini Plan"). Incentive stock options may only be granted to employees. The 2021 Plan is administered by the plan administrator, which is the compensation committee of the Company's board of directors, provided therein, which has discretionary authority, subject only to the express provisions of the 2021 Plan, to interpret the 2021 Plan; determine eligibility for and grant awards; determine form of settlement of awards (whether in cash, shares of stock, other property or a combination of the foregoing), determine, modify or waive the terms and conditions of any award; prescribe forms, rules and procedures; and otherwise do all things necessary to carry out the purposes of the 2021 Plan. The number of shares of common stock reserved for issuance under the 2021 Plan automatically increases on January 1 of each calendar year, starting on January 1, 2022 and continuing through January 1, 2031, in an amount equal to 4% of the total number of shares of the Company's capital stock outstanding on the last day of the calendar month before the date of each automatic increase, or a lesser number of shares determined by the Company's board of directors. As of December 31, 2024, 1,643,165 shares remained available for future issuance under the 2021 Plan.

The exercise price of each stock option granted under the 2021 Plan is 100% of the fair market value of the underlying stock subject to the award, determined as of the date of the grant, or such higher amount as the plan administrator may determine in connection with the grant, and the term of stock option may not be greater than ten years. The vesting and other restrictions are determined at the discretion of the plan administrator with awards generally vesting over a four-year period.

2021 Employee Stock Purchase Plan

In July 2021, Gemini's board of directors approved the 2021 Employee Stock Purchase Plan (the "2021 ESPP"). The first offering period under the 2021 ESPP began on December 1, 2021. The number of shares of common stock reserved for issuance under the 2021 ESPP automatically increases on January 1 of each calendar year, starting on January 1, 2023 and continuing through January 1, 2031, in an amount equal to the least of (a) 1% of the total number of shares of the Company's capital stock outstanding

on the last day of the calendar month before the date of each automatic increase, (b) 43,055 shares of common stock, or (c) such number of shares determined by the Company's board of directors. As of December 31, 2024, 254,926 shares remained available for future issuance under the 2021 ESPP.

2021 Inducement Plan

In February 2021, Gemini's board of directors approved the 2021 Inducement Plan. The 2021 Inducement Plan is a non-stockholder approved stock plan under which equity awards are granted to induce highly-qualified prospective officers and employees who are not currently employed by the Company to accept employment and provide them with a proprietary interest in the Company. From the completion of the merger through December 31, 2024, the Company had not granted awards under the 2021 Inducement Plan. As of December 31, 2024, 161,689 shares remained available for future issuance under the plan.

Out-of-Plan Inducement Grants

From time to time, the Company grants equity awards to newly hired executives as a material inducement to enter into employment with the Company. These grants are made in accordance with Rule 5635(c)(4) of the Nasdaq Listing Rules and are issued outside the 2021 Plan, the 2021 Inducement Plan and each of the other stock incentive plans described above. The inducement grants typically include nonqualified stock options to purchase shares of the Company's common stock, as well as restricted stock unit grants representing shares of the Company's common stock. The option awards have a ten-year term, with 25% of the underlying shares vesting and becoming exercisable on the one-year anniversary of the date of grant, and the balance of each option award vesting in equal monthly installments over 36 months thereafter. The restricted stock unit awards vest with respect to 25% of the underlying shares on each of the first, second, third and fourth anniversaries of the vesting date as set by the Company policy. The inducement grants are included in the stock option and restricted stock unit tables below. During the year ended December 31, 2024, the Company granted 220,000 nonqualified stock options to purchase shares of the Company's common stock, and 146,664 restricted stock units under employment inducement grants. The Company made no employment inducement grants during the year ended December 31, 2023.

Stock Options

For purposes of calculating stock-based compensation, the Company estimates the fair value of stock options using the Black-Scholes option-pricing model. This model incorporates various assumptions, including the expected volatility, expected term, and interest rates.

Prior to the merger, Private Disc lacked company-specific historical and implied volatility information. Therefore, Private Disc estimated its expected stock volatility based on the historical volatility of a publicly traded set of peer public companies and the Company expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the option. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected dividend yield of 0% is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The weighted-average assumptions used to estimate the fair value of stock options granted were as follows:

	Year Ended December 31,	
	2024	2023
Risk-free interest rate	4.00%	4.03%
Expected term (in years)	6.35	6.88
Expected volatility	60%	59%
Expected dividend yield	0%	0%
Fair value per share of common stock	$ 58.99	$ 43.42

The weighted-average grant date fair value of options granted in the years ended December 31, 2024 and 2023 was $35.36 and $26.54 per share, respectively.

The following table summarizes stock option activity for the year ended December 31, 2024.

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (In Years)	Aggregate Intrinsic Value (In Thousands)
Outstanding at December 31, 2023	2,459,037	$ 13.82	7.81	$ 109,078
Granted	896,275	58.99		
Exercised	(315,266)	9.46		
Forfeited	(156,930)	40.46		
Expired	(12,752)	110.25		
Outstanding at December 31, 2024	2,870,364	$ 26.52	7.44	$ 106,766
Exercisable at December 31, 2024	1,589,968	$ 14.72	6.47	$ 77,702

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the common stock as of the end of the period. The aggregate intrinsic value of stock options exercised during the years ended December 31, 2024 and 2023 was $14.5 million and $8.3 million, respectively. The total fair value of options vested during the years ended December 31, 2024 and 2023 was $10.0 million and $3.7 million, respectively. The tax benefit from the exercise of options eligible for a tax deduction realized during the years ended December 31, 2024 and 2023 was $11.0 million and $7.2 million, respectively.

Restricted Stock Units

For purposes of calculating stock-based compensation, the Company determines the fair value of the restricted stock units based on the fair value of the Company's common stock at the time of grant. The following table summarizes restricted stock unit activity for the year ended December 31, 2024.

	Number of Units	Weighted-Average Grant Date Fair Value
Restricted stock units as of December 31, 2023	—	$ —
Granted	507,303	60.26
Forfeited	(32,163)	63.90
Restricted stock units as of December 31, 2024	475,140	$ 60.01

Shares of Restricted Common Stock

As of December 31, 2024, the Company had issued a total of 63,061 shares of restricted common stock to the founders of Private Disc pursuant to subscription agreements and to certain key employees pursuant to the Private Disc Plan at $0.0001 per share. The stock restrictions relate to the sale and transferability of the stock and lapse over the defined vesting period in the restricted stock agreement. The vesting period is generally contingent upon continued employment or consulting services being provided to the Company. In the event of termination, the Company had the right, but not the obligation to repurchase the unvested shares at the original purchase price. As of December 31, 2023, all awards of restricted common stock were fully vested.

A summary of restricted common stock activity was as follows:

	December 31, 2023
Unvested at the beginning of the period	1,916
Vested	(1,916)
Unvested at the end of the period	—

Stock-Based Compensation Expense

Total stock-based compensation expense recorded as research and development and selling, general and administrative expenses, respectively, for employees, directors and non-employees was as follows (in thousands):

	Year Ended December 31,	
	2024	2023
Research and development	$ 7,028	$ 1,767
Selling, general and administrative	9,787	3,763
Total stock-based compensation expense	$ 16,815	$ 5,530

The following table summarizes unrecognized stock-based compensation expense as of December 31, 2024.

	Unrecognized Expense (In Thousands)	Weighted-Average Remaining Period of Recognition (In Years)
Restricted stock units	$ 23,284	3.29
Stock options	28,278	2.85
Total unrecognized equity-based compensation expense	$ 51,562	

13. Income Taxes

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,	
	2024	2023
Federal statutory income tax rate	21.0%	21.0%
State income taxes, net of federal benefit	6.4%	8.0%
Federal and state research and development tax credits	12.1%	4.0%
Stock-based compensation	1.3%	1.3%
Section 162(m) limitation	(1.6)%	(0.8)%
Other	(0.1)%	0.1%
Change in deferred tax asset valuation allowance	(39.4)%	(33.6)%
Effective income tax rate	(0.3)%	0.0%

For the years ended December 31, 2024 and 2023 the Company recorded income tax expense of $0.4 million and $0.1 million, respectively, which was primarily due to state income tax resulting from interest income.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amount of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The significant components of the Company's net deferred income taxes are as follows (in thousands):

	December 31,	
	2024	2023
Deferred tax assets:		
Net operating loss carryforwards	$ 33,209	$ 24,312
Research and experimental expenditures	39,463	20,598
Tax credits	21,248	7,202
Capitalized licenses	6,225	6,890
Accrued expenses	1,137	1,513
Stock-based compensation	3,407	1,234
Operating lease liabilities	447	574
Total deferred tax assets	105,136	62,323
Valuation allowance	(104,670)	(61,768)
Total deferred tax assets, net of valuation allowance	466	555
Deferred tax liabilities:		
Operating right-of-use assets	(418)	(527)
Depreciation	(48)	(28)
Total deferred tax liabilities	(466)	(555)
Net deferred tax assets	$ —	$ —

The Company's income tax provision for the twelve months ended December 31, 2024 related to state and foreign income taxes. The Company has evaluated the positive and negative evidence bearing upon the reliability of its deferred tax assets. Based on this, the Company has provided a valuation allowance for the full amount of the net deferred tax assets as the realization of the deferred tax assets is not determined to be more likely than not. During the year ended December 31, 2024, the valuation allowance increased by $42.9 million primarily due to the increase in the Company's net operating loss, capitalized expenditures and tax credit carryforwards during the period.

As of December 31, 2024, the Company had $117.9 million and $133.8 million of federal and state operating loss carryforwards, respectively. Substantially all of the federal NOLs are not subject to expiration and the state NOLs begin to expire in 2037. These loss carryforwards are available to reduce future federal taxable income, if any. As of December 31, 2024, the

Company also had federal and state research and development tax credit carryforwards of $19.0 million and $2.8 million respectively, to offset future income taxes, which will begin to expire in December 2034. These loss carryforwards are subject to review and possible adjustment by the appropriate taxing authorities.

Utilization of the Company's NOL carryforwards and research and development credit carryforwards may be subject to a substantial annual limitation due to ownership change limitations that have occurred previously or that could occur in the future in accordance with Section 382 as well as similar state provisions. These ownership changes may limit the amount of NOL and research and development credit carryforwards that can be utilized annually to offset future taxable income and taxes, respectively. In general, an ownership change as defined by Section 382 results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50% over a three-year period. Since its formation, the Company has raised capital through the issuance of capital stock on several occasions. These financings could result in a change of control as defined by Section 382. The Company has not yet conducted an analysis under Section 382 to determine if historical changes in ownership through December 31, 2024, would limit or otherwise restrict its ability to utilize its NOL and research and development credit carryforwards. In addition, future changes in ownership occurring after December 31, 2024 could affect the limitation in future years, and any limitation may result in expiration of a portion of the NOL or research and development credit carryforwards before utilization.

The Company generated research and development tax credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development tax credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development tax credit carryforwards and, if an adjustment is required, this adjustment would result in an adjustment to the deferred tax asset established for the research and development tax credit carryforwards and the valuation allowance.

Beginning in 2022, the Tax Cuts and Jobs Act ("TCJA") amended Section 174 and now requires U.S.-based and non-U.S.-based research and experimental expenditures to be capitalized and amortized over a period of five or 15 years, respectively, for amounts paid in tax years starting after December 31, 2021. Prior to the TCJA amendment, Section 174 allowed taxpayers to immediately deduct research and experimental expenditures in the year paid or incurred. The Company has applied this required change in accounting method beginning in 2022 and the computation may be adjusted pending future IRS guidance.

The Company follows the provisions of ASC Topic 740-10, *Accounting for Uncertainty in Income Taxes*, which specifies how tax benefits for uncertain tax positions are to be recognized, measured, and recorded in financial statements; requires certain disclosures of uncertain tax matters; specifies how reserves for uncertain tax positions should be classified on the consolidated balance sheets; and provides transition and interim period guidance, among other provisions. As of December 31, 2024 and 2023, the Company has not recorded any amounts for uncertain tax positions. The Company's policy is to recognize interest and penalties accrued on any uncertain tax positions as a component of income tax expense, if any, in its consolidated statements of operations and comprehensive loss. As of December 31, 2024 and 2023, the Company had no reserves for uncertain tax positions. For the years ended December 31, 2024 and 2023, no estimated interest or penalties were recognized on uncertain tax positions.

The Company files federal income tax returns in the United States, the Netherlands and Australia and state income tax returns in Massachusetts and various other state jurisdictions. The Company's tax returns for the years ended December 31, 2019 to December 31, 2024 remain open and subject to examination by the Internal Revenue Service and state taxing authorities.

14. Net Loss Per Share

Basic and diluted net loss per share is computed by dividing net loss by the weighted-average common shares outstanding. The weighted-average common shares outstanding used in the basic and diluted net loss per share calculation includes the pre-funded warrants issued in connection with the Company's follow-on public offering in June 2023 and registered direct offering in February 2023 as the pre-funded warrants are exercisable for nominal cash consideration. In September 2023, 1,229,224 of the pre-funded warrants were exercised in a cashless transaction which resulted in 1,229,221 shares of common stock being issued to the investor. As of December 31, 2024, 204,081 pre-funded warrants were outstanding. The following table sets forth the computation of the Company's basic and diluted net loss per share (in thousands, except share and per share data):

	Year Ended December 31,	
	2024	2023
Numerator:		
Net loss	$ (109,357)	$ (76,429)
Denominator:		
Weighted-average common shares outstanding, basic and diluted	27,606,022	22,315,877
Net loss per share, basic and diluted	$ (3.96)	$ (3.42)

The Company has generated a net loss in all periods presented, so the basic and diluted net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive. The Company excluded the following potential common shares from the computation of diluted net loss per share because including them would have had an anti-dilutive effect:

	December 31,	
	2024	2023
Restricted stock units	475,140	—
Options to purchase common stock	2,870,364	2,459,037
Employee stock purchase program	9,003	4,878

15. Commitments and Contingencies

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to its vendors, lessors, contract research organizations, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its Board that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. The Company has not incurred any material costs as a result of such indemnifications and is not currently aware of any indemnification claims.

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the years ended December 31, 2024 and 2023 and, to the best of its knowledge, no material legal proceedings are currently pending or threatened.

Payments Upon Termination

The Company enters into contracts in the normal course of business with contract research organizations ("CROs"), contract development and manufacturing organizations ("CDMOs") and other third parties for preclinical studies, clinical trials and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancelable by the Company upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of the Company's service providers, up to the date of cancellation and, in the case of certain arrangements with CROs and CDMOs, may include noncancelable fees. Under such agreements, the exact amounts owed by the Company in the event of termination will be based on the timing of the termination and the exact terms of the agreement. As of December 31, 2024, the Company has not recognized any amounts related to these contingencies as the amount and timing of such payments are not fixed and estimable.

16. Related Party Transactions

In June 2024, certain existing investors participated in the Company's underwritten offering (see *Note 11 - Stockholders' Equity*).

In June 2023, an existing investor participated in the Company’s follow-on offering (see *Note 11 - Stockholders' Equity*).

In March 2023, the Company executed a promissory note for an aggregate principal amount of $0.5 million from an existing investor. The Company did not use these funds and repaid the note four days later, recording a de minimis amount of interest expense based on the then Federal funds rate for short term loans of 4.5% per annum.

In February 2023, certain existing investors participated in the Company’s registered direct offering (see *Note 11 - Stockholders' Equity*).

The landlord of the Company’s leased office space in Watertown, Massachusetts is a related party of the Company due to its equity ownership.

17. Segment Information

The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions, resulting in a single reportable segment. The Company has determined that its Chief Operating Decision Maker

("CODM") is its Chief Executive Officer. The Company's CODM reviews the Company's financial information on a consolidated basis for purposes of allocating resources and assessing financial performance.

The Company has assembled a portfolio of clinical and preclinical product candidates that aim to modify fundamental biological pathways associated with the formation and function of red blood cells, specifically heme biosynthesis and iron homeostasis. The Company has not generated any revenue since its inception and does not expect to generate any revenue from the sale of products in the near future. The Company primarily incurs expenses in connection with the research and development of its product candidates as well as selling, general and administrative costs consisting of salaries and related costs for personnel in executive, finance, commercial, corporate and business development, and administrative functions, as well as legal, consulting, commercial planning, market research and professional fees.

The key measure of segment profit or loss that the CODM uses to allocate resources and assess performance is the Company's consolidated net loss, as reported on the consolidated statements of operations and comprehensive loss. In addition, the CODM is regularly provided the following significant segment expense categories which are reviewed against budgeted expectations to assist in resource allocation decision-making:

	Year Ended December 31,	
	2024	2023
Bitopertin program external expense	$ (28,105)	$ (17,271)
DISC-0974 program external expense	(16,943)	(9,136)
DISC-3405 program external expense	(13,934)	(20,168)
Other external research and development expense	(6,879)	(4,752)
Internal and other research and development expense	(23,782)	(16,170)
Selling, general and administrative expense	$ (23,262)	$ (18,098)
Stock-based compensation expense	$ (16,815)	$ (5,530)
Other segment items*	$ 20,363	$ 14,696
Net loss	$ (109,357)	$ (76,429)

*Other segment items include interest income, interest expense, other expense, and income tax expense as reported on the consolidated statements of operations and comprehensive loss.

Assets regularly provided to the CODM are consistent with those reported on the consolidated balance sheets with particular emphasis on the Company's available liquidity, including its cash, cash equivalents and marketable securities balances. All of the Company's tangible assets are held in the United States.

18. Subsequent Events

Milestone Payment

In January 2025, the Company dosed the first patient in the Phase 2 clinical trial of DISC-0974. Per the license agreement, a milestone payment of $3.0 million was paid to AbbVie on January 31, 2025. For additional details on the license agreement with AbbVie, refer to *Note 10 - Development and License Agreements*.

Underwritten Offering

On January 22, 2025, the Company entered into an Underwriting Agreement (the "Underwriting Agreement") with Jefferies LLC, Leerink Partners LLC, Stifel, Nicolaus & Company, Incorporated and Cantor Fitzgerald & Co. as representatives of the several underwriters listed on Schedule I thereto (the "Underwriters") related to an underwritten offering of (i) 3,918,182 shares of common stock of the Company, par value $0.0001 per share ("Common Stock") at a price to the public of $55.00 per share and (ii) pre-funded warrants to purchase an aggregate of 181,818 shares of Common Stock (the "Pre-Funded Warrants"), at a price to the public of $54.9999 per Pre-Funded Warrant, which represents the per share public offering price for the Common Stock less the $0.0001 per share exercise price for each such Pre-Funded Warrant. In addition, the Company granted the Underwriters an option exercisable for 30 days from the date of the underwriting agreement to purchase, at the public offering price less any underwriting discounts

and commissions, up to an additional 615,000 shares of Common Stock, which the Underwriters exercised in full on January 23, 2025.

The net proceeds from the Offering are expected to be approximately $243.3 million, which includes the proceeds from the Underwriters' exercise in full of their option to purchase additional shares, after deducting the underwriting discount and estimated offering expenses. The Company will receive nominal proceeds, if any, from the exercise of the Pre-Funded Warrants.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our Principal Executive Officer (our Chief Executive Officer) and Principal Financial Officer (our Chief Financial Officer), has evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2024, our Principal Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were not effective at the reasonable assurance level due to the material weakness in the design and maintenance of effective Information Technology General Controls ("ITGC") over certain key financial IT systems described below.

Material Weakness

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company's annual or interim financial statements will not be prevented or detected on a timely basis.

We identified a material weakness related to a lack of design and maintenance of effective ITGC's over certain key financial IT systems. Consequently, our IT application controls and IT dependent manual controls that rely upon the ineffective ITGC's, or that use information from the systems impacted by the ineffective ITGC's were also deemed ineffective because they could have been adversely impacted. This material weakness did not result in any identified misstatements to our financial statements.

Notwithstanding the material weaknesses in our internal control over financial reporting, our management has concluded that our consolidated financial statements and related notes thereto included in this Annual Report fairly present in all material respects the financial condition, results of operations and cash flows of the Company and have been prepared in accordance with generally accepted accounting principles. Our Principal Executive Officer and Principal Financial Officer have certified that, based on each such officer's knowledge, the financial statements, as well as the other financial information included in this Annual Report, fairly present in all material respects the financial condition, results of operations and cash flows of the Company as of, and for, the periods presented in this Annual Report. Ernst & Young LLP has issued an unqualified opinion on our financial statements, which is included in Part IV of this Annual Report on Form 10-K.

Management's Plan for Remediation of the Material Weakness

Management, with the oversight of the audit committee of the board of directors, is committed to maintaining a strong internal control environment. In response to the material weakness identified above, we have identified and begun to implement remediation efforts which include: (i) developing and implementing additional training and awareness programs addressing ITGCs and policies, including educating control owners concerning the principles and requirements of each control, with a focus on understanding user access and program changes; (ii) engaging third-party consultants to assist with remediation efforts, including enhancing our risk assessment, evaluating gaps in our current processes and controls, and developing a remediation plan; (iii) expanding available resources through internal hiring and; (iv) enhancing quarterly reporting on the remediation measures to the Audit Committee of the Board of Directors.

We believe that these actions, when fully implemented, will remediate the material weakness. However, the material weakness will not be considered fully remediated until management designs and implements effective controls that operate for a sufficient period of time and management has concluded, through testing, that these controls are effective. As we continue to evaluate operating effectiveness and monitor improvements to our internal control over financial reporting, we may take additional measures to address control deficiencies or modify the remediation plan described above.

Internal Control Over Financial Reporting

Management's Report on Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rule 13a-15(f) under the Exchange Act. We maintain a system of internal control that is designed to provide reasonable assurance regarding the reliability of our financial reporting and the preparation of consolidated financial statements for external purposes in accordance with generally accepted accounting principles.

Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2024. In making this assessment, it used the criteria established in Internal Control—Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on such assessment, and as a result of the material weakness described above, our Principal Executive Officer and Principal Financial Officer concluded that, as of December 31, 2024, our disclosure controls and procedures were not effective at the reasonable assurance level. However, after giving full consideration to this material weakness, our management has concluded that our consolidated financial statements present fairly, in all material respects, our financial position, results of operations and cash flows for the periods disclosed in conformity with U.S. GAAP.

Changes in Internal Control Over Financial Reporting

Other than as discussed above, there have not been any changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the twelve months ended December 31, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Disc Medicine, Inc.

Opinion on Internal Control Over Financial Reporting

We have audited Disc Medicine, Inc.'s internal control over financial reporting as of December 31, 2024, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, because of the effect of the material weakness described below on the achievement of the objectives of the control criteria, Disc Medicine, Inc. (the Company) has not maintained effective internal control over financial reporting as of December 31, 2024, based on the COSO criteria.

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the company's annual or interim financial statements will not be prevented or detected on a timely basis. The following material weakness has been identified and included in management's assessment. Management has identified a material weakness related to ineffective information technology general controls, or ITGCs, due to a lack of design and maintenance of effective user access and program change management controls over certain key financial IT systems. Consequently, the related IT application controls and IT dependent manual controls that are dependent on or use information from the systems impacted, were also deemed ineffective.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, and the related notes. This material weakness was considered in determining the nature, timing and extent of audit tests applied in our audit of the 2024 consolidated financial statements, and this report does not affect our report dated February 27, 2025, which expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and

performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Boston, Massachusetts

February 27, 2025

ITEM 9B. OTHER INFORMATION

The following table discloses any officer (as defined in Rule 16a-1(f) under the Exchange Act) or director who entered into, modified or terminated a Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement (as such terms are defined in Item 408 of Regulation S-K) during the three months ended December 31, 2024:

Name and Title	Type of Trading Arrangement	Action Taken (Date of Action)	Duration or End Date	Aggregate Number of Securities to be Sold	Description of Trading Arrangement
William Savage, M.D., Ph.D. Chief Medical Officer	Trading plan intended to satisfy the affirmative defense conditions of Securities Exchange Act Rule 10b5-1(c)	Adoption (November 18, 2024)	End Date (May 30, 2025)	Up to 21,870	Sale

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required under this item is incorporated herein by reference to our definitive proxy statement pursuant to Regulation 14A, which proxy statement will be filed with the Securities and Exchange Commission, or SEC, not later than 120 days after the close of our fiscal year ended December 31, 2024.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this item regarding executive compensation will be included in our definitive proxy statement for our 2025 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days after December 31, 2024, and such information is incorporated herein by reference (excluding pay versus performance disclosure).

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The information required by this item regarding security ownership of certain beneficial owners and management and related stockholder matters will be included in our definitive proxy statement for our 2025 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days after December 31, 2024, and such information is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The information required by this item regarding certain relationships and related transactions, and director independence will be included in our definitive proxy statement for our 2025 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days after December 31, 2024, and such information is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Our independent public accounting firm is Ernst & Young, LLP, Boston, MA, PCAOB Auditor ID: 42.

The information required by this item will be included in our definitive proxy statement for our 2025 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days after December 31, 2024, and such information is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

1. Financial Statements

For a list of the financial statements included herein, see Index to the Consolidated Financial Statements on page 112 of this Annual Report on Form 10-K, incorporated into this Item by reference.

2. Financial Statement Schedules

Financial statement schedules have been omitted because they are either not required or not applicable or the information is included in the consolidated financial statements or the notes thereto.

3. Exhibits

The exhibits required by Item 601 of Regulation S-K and Item 15(b) of this Annual Report on Form 10-K are listed in the Exhibit Index immediately preceding the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index are incorporated by reference herein.

Exhibit Index

Exhibit Number	Description
2.1†	Agreement and Plan of Merger and reorganization, dated as of August 9, 2022, by and among Gemini Therapeutics, Inc. Gemstone Merger Sub, Inc. and Disc Medicine, Inc. (incorporated by reference to Exhibit 2.1 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).
3.1	Amended and Restated Certificate of Incorporation of Disc Medicine, Inc. (incorporated by reference to Annex B to Gemini Therapeutics, Inc.'s Proxy Statement/Prospectus on Form S-4/A (Registration No. 333-249785)).
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Disc Medicine, Inc., dated December 28, 2022 (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Disc Medicine, Inc., dated December 29, 2022 (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
3.4	Amended and Restated By-laws of Disc Medicine, Inc. (incorporated by reference to Annex C to Gemini Therapeutics, Inc.'s Proxy Statement/Prospectus on Form S-4/A (Registration No. 333-249785)).
4.1	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Exchange Act (incorporated by reference to Exhibit 4.4 to Gemini Therapeutics, Inc.'s Annual Report on Form 10-K filed on March 10, 2022).
4.2	Registration Rights Agreement, among Disc Medicine, Inc. and certain of its stockholders, dated December 28, 2022 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
4.3	Registration Rights Agreement, dated February 5, 2021, by and among Gemini Therapeutics, Inc. and the stockholder parties thereto (incorporated by reference to Exhibit 10.1 on Form 8-A12B/A filed on February 5, 2021).
4.4	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-39438) filed February 14, 2023).
4.5	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-39438) filed on January 24, 2025).
10.1††	License Agreement by and among Disc Medicine, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated May 7, 2021 (incorporated by reference to Exhibit 10.2 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).
10.2††	License Agreement by and between Disc Medicine, Inc. and AbbVie Deutschland GmbH & Co, KG, dated September 13, 2019 (incorporated by reference to Exhibit 10.3 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on November 23, 2022).
10.3††	Exclusive License Agreement, dated January 19, 2023, by and between Disc Medicine, Inc. and Mabwell Therapeutics, Inc. (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-39438) filed on May 15, 2023)
10.3	Lease by and between Disc Medicine, Inc. and ARE-MA Region No. 75, LLC, dated October 29, 2021 (incorporated by reference to Exhibit 10.4 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).
10.4	Addendum to License Agreement by and among Disc Medicine, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated December 7, 2021 (incorporated by reference to Exhibit 10.5 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).

Exhibit Number	Description
10.5	Amendment to Addendum to License Agreement by and among Disc Medicine, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated February 28, 2022 (incorporated by reference to Exhibit 10.6 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).
10.6	Second Amendment to Addendum to License Agreement by and among Disc Medicine, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated May 31, 2022 (incorporated by reference to Exhibit 10.7 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on September 2, 2022).
10.7	Third Amendment to Addendum to License Agreement by and among Disc Medicine, Inc., F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., dated October 19, 2022 (incorporated by reference to Exhibit 10.21 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on November 3, 2022).
10.8	Common Stock Issuance Agreement, dated as of December 29, 2022, by and between Disc Medicine Opco, Inc., F. Hoffmann-La Roche Ltd. and Hoffmann-La Roche Inc. (incorporated by reference to Exhibit 10.5 to the Registrant's Current Report on Form 8-K filed on December 29, 2022)
10.9††	Stock Purchase and Restriction Agreement, dated September 13, 2019, by and between Disc Medicine, Inc. and AbbVie Deutschland GmbH & Co. KG (incorporated by reference to Exhibit 10.22 to the Registrant's Proxy Statement/Prospectus on Form S-4 filed on November 23, 2022).
10.10#	Disc Medicine, Inc. Amended and Restated 2021 Stock Option and Incentive Plan (incorporated by reference to Exhibit 99.1 of Disc Medicine, Inc.'s Form S-8 filed on January 18, 2023 (Registration No. 333-269271)).
10.11#	Forms of Award Agreements under the Disc Medicine, Inc. Amended and Restated 2021 Stock Option and Incentive Plan (incorporated by reference to Exhibit 99.2 of Disc Medicine, Inc.'s Form S-8 filed on January 18, 2023 (Registration No. 333-269271)).
10.12#	Disc Medicine, Inc. Amended and Restated 2021 Employee Stock Purchase Plan (incorporated by reference to Exhibit 99.3 of Disc Medicine, Inc.'s Form S-8 filed on January 18, 2023 (Registration No. 333-269271)).
10.13#	2017 Stock Option and Grant Plan of Disc Medicine, Inc., and form of award agreements thereunder (incorporated by reference to Exhibit 10.9 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
10.14#	2021 Inducement Plan (incorporated by reference to Exhibit 99.3 of Gemini Therapeutics, Inc.'s Form S-8 filed on April 13, 2021 (Registration No. 333-255194)).
10.15#	Form of Indemnification Agreement for Directors of Disc Medicine, Inc. (incorporated by reference to Exhibit 10.3 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
10.16#	Form of Indemnification Agreement for Officers of Disc Medicine, Inc. (incorporated by reference to Exhibit 10.4 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
10.17#	Employment Agreement, dated as of December 29, 2022, by and between Disc Medicine, Inc. and John Quisel, J.D. Ph.D. (incorporated by reference to Exhibit 10.6 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
10.18#	Transition and Separation Agreement, dated as of November 27, 2023, by and between Disc Medicine, Inc. and Joanne Bryce. (incorporated by reference to Exhibit 10.18 to the Registrant's Annual Report on Form 10-K (File No. 001-39438) filed on March 21, 2024).
10.19#	Employment Agreement, dated as of December 29, 2022, by and between Disc Medicine, Inc. and William Savage, M.D., Ph.D. (incorporated by reference to Exhibit 10.19 to the Registrant's Annual Report on Form 10-K (File No. 001-39438) filed on March 31, 2023).
10.20#	Employment Agreement, dated as of December 29, 2022, by and between Disc Medicine, Inc. and Jonathan Yu (incorporated by reference to Exhibit 10.20 to the Registrant's Annual Report on Form 10-K (File No. 001-39438) filed on March 31, 2023).

Exhibit Number	Description
10.21#	Employment Agreement, dated as of February 7, 2024, by and between Disc Medicine, Inc. and Jean M. Franchi (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-39438) filed on February 7, 2024).
10.22#	Form of Non-Qualified Stock Option Agreement Non-Plan Inducement Grant Restricted Stock Unit Award Agreement Non-Plan Inducement Grant (incorporated by reference to Exhibit 99.5 to the Registrant's Registration Statement on Form S-8 (File No 333-278129) filed on March 21, 2024).
10.23#	Form of Restricted Stock Unit Award Agreement Non-Plan Inducement Grant (incorporated by reference to Exhibit 99.6 to the Registrant's Registration Statement on Form S-8 (File No 333-278129) filed on March 21, 2024).
10.24	Second Amendment to Lease, dated August 29, 2024, by and between Disc Medicine, Inc. and ARE-MA Region No. 75. LLC (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed on November 12, 2024).
10.25†+	Loan and Security Agreement, dated as of November 6, 2024, by and among Disc Medicine, Inc., the other Borrower party thereto, the Lenders party thereto, and Hercules Capital, Inc., as Agent (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K (File No. 001-39438) filed on November 8, 2024).
10.26	Controlled Equity Offering℠ Sales Agreement, dated November 15, 2024, by and between Disc Medicine, Inc. and Cantor Fitzgerald & Co. (incorporated by reference to Exhibit 1.1 to the Registrant's Current Report on Form 8-K (File No. 001-39438) filed on November 15, 2024).
10.27*†	Side Letter Agreement dated December 24, 2024 by and among Disc Medicine, Inc., Mabwell Therapeutics, Inc. and Mabwell (Shanghai) Bioscience, Inc.
14.1	Code of Business Conduct and Ethics of Disc Medicine, Inc. (incorporated by reference to Exhibit 14.1 to the Registrant's Current Report on Form 8-K filed on December 29, 2022).
19.1*	Insider Trading Policy
21.1*	List of Subsidiaries of Disc Medicine, Inc.
23.1*	Consent of Ernst & Young LLP, independent registered accounting firm.
31.1*	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
97.1*	Compensation Recovery Policy
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.
** Furnished herewith.
# Indicates a management contract or compensatory plan.
† The annexes, schedules, and certain exhibits to the Merger Agreement have been omitted pursuant to Item 601(b)(2) of Regulation S-K. Gemini hereby agrees to furnish supplementally a copy of any omitted annex, schedule or exhibit to the Commission upon request.

†† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with the rules of the Securities and Exchange Commission.
+ Portions of this exhibit are redacted in accordance with Item 601(b)(10)(iv) of Regulation S-K.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

DISC MEDICINE, INC.

By: /s/ Jean Franchi

Date: February 27, 2025

Jean Franchi
Chief Financial Officer
(Principal Financial and Accounting Officer)

POWER OF ATTORNEY

Each person whose signature appears below constitutes and appoints each of John Quisel, J.D., Ph.D., Jean Franchi and Rahul Khara, Pharm.D., J.D., acting alone or together with another attorney-in-fact, as his or her true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for such person and in his or her name, place and stead, in any and all capacities, to sign any or all further amendments to this annual report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorney-in-fact and agent, or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ John Quisel John Quisel, J.D., Ph.D.	President, Chief Executive Officer and Director (Principal Executive Officer)	February 27, 2025
/s/ Jean Franchi Jean Franchi	Chief Financial Officer (Principal Financial and Accounting Officer)	February 27, 2025
/s/ Donald Nicholson Donald Nicholson, Ph.D.	Director	February 27, 2025
/s/ Kevin Bitterman Kevin Bitterman, Ph.D.	Director	February 27, 2025
/s/ Liam Ratcliffe Liam Ratcliffe, M.D., Ph.D.	Director	February 27, 2025
/s/ William White William White MPP, J.D.	Director	February 27, 2025
/s/ Mona Ashiya Mona Ashiya, Ph.D.	Director	February 27, 2025
/s/ Mark Chin Mark Chin, MS, MBA	Director	February 27, 2025
/s/ Georges Gemayel Georges Gemayel, Ph.D.	Director	February 27, 2025

Exhibit 10.27

Certain identified information has been excluded from this exhibit because it is both not material and is the type that the registrant treats as private or confidential. Information that was omitted has been noted in this document with a placeholder identified by the mark "[***]".

Confidential

SIDE LETTER AGREEMENT

This Side Letter Agreement ("**Side Letter**") is made and entered into as of December 24, 2024 ("**Side Letter Execution Date**") by and among **Disc Medicine, Inc.**, a Delaware corporation having its registered address at 321 Arsenal Street, Suite 101, Watertown, MA 02472 ("**Disc**"), on the one hand, and **Mabwell Therapeutics, Inc.**, a California corporation having its registered address at 12250 El Camino Real, Suite 140, San Diego, CA 92130 ("**Mabwell Therapeutics**") and Mabwell Therapeutics's Affiliate, **Mabwell (Shanghai) Bioscience Co. Ltd.**, a Chinese corporation having its registered address at 576 Libing Road, ZhangJiang Creative Park, Building 3, Floor 4 , Pudong New District, Shanghai 201210, China ("**Mabwell Shanghai**" and, together with Mabwell Therapeutics, "**Mabwell**"), on the other hand. Disc and Mabwell are sometimes referred to herein individually as a "**Party**" and collectively as the "**Parties**".

Recitals

WHEREAS, Disc and Mabwell Therapeutics entered into a certain Exclusive License Agreement ("**License Agreement**") on January 19, 2023 ("**License Agreement Effective Date**") pursuant to which Mabwell Therapeutics granted to Disc certain intellectual property rights of Mabwell in the Licensed Territory (as defined therein);

WHEREAS, Disc and Mabwell Shanghai have engaged in joint research pursuant to the License Agreement that has resulted in certain intellectual property [***] (such intellectual property, "**Subject IP**" and Patents claiming or directed to such intellectual property "**Subject Patents**"); and

WHEREAS, effective *nunc pro tunc* as of the License Agreement Effective Date, the Parties desire to clarify certain items relating to the Subject IP.

NOW, THEREFORE, in consideration of the premises and the mutual promises and conditions set forth herein and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties, intending to be legally bound, do hereby agree as follows:

1. Capitalized terms used in this Side Letter shall have the meanings set forth in the License Agreement, unless otherwise defined in this Side Letter.

2. The Subject IP is Joint Improvement IP as specified in Section 7.1(b)(iii) of the License Agreement, and Mabwell grants to Disc a license under the Subject IP as part of the Licensed IP in accordance with Sections 1.89, 5.1, and 9.2(a) of the License Agreement.

3. The Subject Patents are Joint Improvement Patents and the Parties agree that Subject Patents shall be deemed Core Licensed Patents, thus, pursuant to Section 7.2(a) and Section 7.3(b) of the License Agreement, (a) Disc has the right to prosecute, maintain, enforce, and defend the Subject Patents in the Licensed Territory, and (b) Mabwell has the right to prosecute, maintain, enforce, and defend the Subject Patents in the Mabwell Territory, in each case ((a) and (b)), all in accordance with, and subject to, Sections 7.2,7.3, and 13.13 of the License Agreement.

4. This Side Letter shall be governed by the laws of the State of New York, excluding its conflicts of laws principles, and Section 13.5 of the License Agreement shall apply to any disputes arising hereunder. This Side Letter may not be modified except by a written instrument signed by both Parties. No failure or delay of one of the Parties to insist upon strict performance of any of its rights or powers under this Side Letter shall operate as a waiver thereof, nor shall any other single or partial exercise of such right or power preclude any other further exercise of any rights or remedies provided by law. No waiver by a Party of a particular right or remedy shall be effective unless in writing and signed by an authorized representative of such Party. This Side Letter (together with the License Agreement) (a) constitutes the complete, final and exclusive agreement and all the covenants, promises, agreements, warranties, representations, conditions and understandings among the Parties with respect to the subject matter hereof and thereof and (b) supersedes all prior and contemporaneous agreements and understandings among the Parties with respect to the subject matter hereof and thereof. This Side Letter may be executed in multiple counterparts (including by facsimile), each of which shall be deemed an original, all of which together shall constitute one and the same instrument and such signatures shall be deemed to bind each Party as if they were original signatures.

[*Signature Page Follows*]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have executed this Side Letter Agreement by their authorized representatives as of the Side Letter Execution Date.

DISC MEDICINE, INC.

By: /s/ John Quisel

Name: John Quisel

Title: CEO

MABWELL THERAPEUTICS, INC.

By: /s/ Xin Du, PhD

Name: Xin Du, PhD

Title: Chief Executive Officer

MABWELL (SHANGHAI) BIOSCIENCE CO., LTD.

By: /s/ Datao Liu

Name: Datao Liu

Title: CEO

Exhibit 19.1

DISC MEDICINE, INC.

INSIDER TRADING POLICY

Disc Medicine, Inc. (the "Company") has adopted the following policy and procedures for securities trading by Company directors and employees (our "Insider Trading Policy"). Our Insider Trading Policy is intended to prevent the misuse of material nonpublic information, insider trading in securities, and the severe consequences associated with violations of insider trading laws. It is your obligation to review, understand, and comply with this Insider Trading Policy and applicable laws. Our Board of Directors has approved this Insider Trading Policy, and we have appointed Rahul Khara, the Company's General Counsel (with their designees, the "General Counsel") to administer the policy and to be available to answer your questions.

PART I. OVERVIEW

A. Who Must Comply?

This Insider Trading Policy applies to all of our employees and members of our Board of Directors and certain designated consultants, including anyone employed by or acting as a director of any of the Company's subsidiaries, as well as any other individuals whom the General Counsel may designate as Insiders (defined below) because they have access to material nonpublic information about the Company.

In addition, all of our directors, executive officers (as defined by Section 16 of the Securities Exchange Act of 1934, as amended (the "Exchange Act")) and other designated employees and certain consultants must comply with the Trading Procedures included in Part II of this Insider Trading Policy (the "Trading Procedures"); we will refer to these individuals in this policy as "Insiders." The Trading Procedures provide rules for when Insiders can trade in our securities and explain the process for mandatory pre-clearance of proposed trades. You will be notified if you are considered to be an Insider who is required to comply with the Trading Procedures.

This Insider Trading Policy and, for Insiders, the Trading Procedures also apply to the following persons ("Affiliated Persons"):

- your "Family Members" ("Family Members" are (a) your spouse or domestic partner, children, stepchildren, grandchildren, parents, stepparents, grandparents, siblings and in-laws who reside in the same household as you, (b) your children or your spouse's children who do not reside in the same household as you but are financially dependent on you, (c) any of your other family members who do not reside in your household but whose transactions are directed by you, and (d) any other individual over whose account you have control and to whose financial support you materially contribute. (Materially contributing to financial support would include, for example, paying an individual's rent but not just a phone bill.).);

- all trusts, family partnerships and other types of entities formed for your benefit or for the benefit of a member of your family and over which you have the ability to influence or direct investment decisions concerning securities;

- all persons who execute trades on your behalf; and

- all investment funds, trusts, retirement plans, partnerships, corporations and other types of entities over which you have the ability to influence or direct investment decisions concerning securities; provided, however, that the Trading Procedures do not apply to any such entity that engages in the investment of securities in the ordinary course of its business (e.g., an investment fund, venture fund or partnership) if the entity has established its own insider trading controls and procedures in compliance with applicable securities laws and it and/or its affiliated entities: (a) engage in the investment of securities in the ordinary course of their respective businesses; (b) have established insider trading controls and procedures in compliance with securities laws; and (c) are aware the securities laws prohibit any person or entity who has material nonpublic information concerning the Company from purchasing or selling securities of the Company or from communicating such information to any other person under circumstances in which it is reasonably foreseeable that such person is likely to purchase or sell securities.

You are responsible for ensuring compliance with this Insider Trading Policy, including the Trading Procedures contained herein, by all of your Affiliated Persons.

B. What is Prohibited by this Insider Trading Policy?

You and your Affiliated Persons are prohibited from engaging in insider trading and from trading in securities in violation of this Insider Trading Policy. "Insider trading" is (1) trading (buying or selling) the securities of a company whether for your account or for the account of another, while in the possession of material nonpublic information (see definition below) about that company or (2) disclosing material nonpublic information about a company to others who may trade on the basis of that information. Insider trading can result in criminal prosecution, jail time, significant fines and public embarrassment for you and the Company.

Prohibition on Trading in Company Securities

When you are in possession of material nonpublic information about the Company, whether positive or negative, you are prohibited from trading (whether for your account or for the account of another) in the Company's securities, which include common stock, options to purchase common stock, any other type of securities that the Company may issue (such as preferred stock, convertible debentures, warrants and exchange-traded options), and any derivative securities that provide the economic equivalent of ownership of any of the Company's securities or an opportunity, direct or indirect, to profit from any change in the value of the Company's securities, except for trades made pursuant to plans approved by the General Counsel in accordance with this policy that are intended to comply with Rule 10b5-1 under the Exchange Act.

The trading prohibitions in this Insider Trading Policy do not apply to: (1) an exercise of an employee stock option when payment of the exercise price is made in cash or (2) the withholding by the Company of shares of stock upon vesting of restricted stock or upon settlement of restricted stock units to satisfy applicable tax withholding requirements if (a) such withholding is required by the applicable plan or award agreement or (b) the election to exercise such tax withholding right was made by the Insider in compliance with the Trading Procedures.

The trading prohibitions in this Insider Trading Policy do apply, however, to the use of outstanding Company securities to pay part or all of the exercise price of a stock option, any sale of stock as part of a broker-assisted cashless exercise of an option, and any other market sale for the purpose of generating the cash needed to pay the exercise price of an option.

Prohibition on Tipping

Providing material nonpublic information about the Company to another person who may trade or advise others to trade on the basis of that information is known as "tipping" and is illegal. You are prohibited from providing material nonpublic information about the Company to a friend, relative, or anyone else who might buy or sell a security or other financial instrument on the basis of that information, whether or not you intend to or actually do realize a profit (or any other benefit) from such tipping. Additionally, you are prohibited from recommending to any person that such person engage in or refrain from engaging in any transaction involving the Company's securities, or otherwise give trading advice concerning the Company's securities, if you are in possession of material nonpublic information about the Company.

Prohibition on Trading in Securities of Other Companies

Whenever, during the course of your service to or employment by the Company, you become aware of material nonpublic information about another company (1) with which the Company has an existing business relationship, including but not limited to, the Company's distributors, vendors, customers or suppliers or collaboration, marketing, research, development or licensing partners, or (2) with which the Company is in active discussions concerning a potential transaction or business relationship, neither you nor your Affiliated Persons may trade in any securities of that company, give trading advice about that company, tip or disclose that information, pass it on to others, or engage in any other action to take advantage of that information.

If your work regularly involves handling or discussing confidential information of companies in either of the foregoing categories, you should consult with the General Counsel before trading in any of those company's securities.

Additionally, if you believe you may be in possession of nonpublic information about the Company that could potentially have a material effect on the stock price of a company with which the Company does not have an existing business relationship or with which the Company is not discussing a potential transaction or business relationship, you should exercise caution when trading in the securities of that company because the U.S. Securities and Exchange Commission (the "SEC") has successfully brought an insider trading claim against an insider in those circumstances.

Duration of Trading Prohibitions

These trading prohibitions continue whenever and for as long as you know or are in possession of material nonpublic information. Remember, anyone scrutinizing your transactions will be doing so after the fact, with the benefit of hindsight. As a practical matter, before engaging in any transaction, you should carefully consider even the appearance of improper insider trading and how enforcement authorities and others might view the transaction in hindsight.

This Insider Trading Policy applies to you and your Affiliated Persons so long as you are associated with the Company. If you leave the Company for any reason, this Insider Trading Policy, including, if applicable, the Trading Procedures described in Part III, will continue to apply to you and your Affiliated Persons until the first trading day after any material nonpublic information known to you has become public or is no longer material.

C. What is Material Nonpublic Information?

This Insider Trading Policy prohibits you from trading in a company's securities if you are in possession of information about the company that is both "*material*" and "*nonpublic*." If you have a question whether certain information you are aware of is material or has been made public, you should consult with the General Counsel.

"Material" Information

Information about our Company or any other company is "material" if it could reasonably be expected to affect the investment decisions of a stockholder or potential investor or if disclosure of the information could reasonably be expected to significantly alter the total mix of information in the marketplace about us or any other company. We speak mostly in this Insider Trading Policy about determining whether information about us is material and nonpublic, but the same analysis applies to information about other companies covered by this policy that would preclude you from trading in their securities.

In simple terms, material information is any type of information that could reasonably be expected to affect the market price of our securities. Both positive and negative information may be material. While it is not possible to identify all information that would be deemed "material," the following items are examples of the types of information that could be material:

- developments regarding any programs in preclinical or clinical development, including recent regulatory interaction and/or data that have been recently generated from ongoing or recently completed preclinical or clinical trials;
- developments regarding the intellectual property and/or freedom to operating for any of the current programs or product candidates under development;
- projections of future earnings or losses, or other earnings guidance;
- quarterly financial results that are known but have not been publicly disclosed;
- potential restatements of the Company's financial statements, changes in auditors or auditor notification that the Company may no longer rely on an auditor's audit report;

- pending or proposed corporate mergers, acquisitions, tender offers, joint ventures or dispositions of significant assets;
- changes in senior management or member of our Board of Directors;
- significant actual or threatened litigation or governmental investigations or major developments in such matters;
- cybersecurity risks and incidents, including the discovery of significant vulnerabilities or breaches;
- significant developments regarding products, customers, suppliers, orders, contracts or financing sources (e.g., the acquisition or loss of a contract);
- changes in dividend policy, declarations of stock splits, or proposed securities offerings or other financings;
- potential defaults under our credit agreements or indentures or potential material liquidity issues; and
- bankruptcies or receiverships.

The above items will not always be material. For example, some new products or contracts may clearly be material while others may not be. No "bright-line" standard or list of items can adequately address the range of situations that may arise; information and events should be carefully considered in terms of their materiality to the Company.

"Nonpublic" Information

Material information is "nonpublic" if it has not been disseminated in a manner making it available to investors generally.

To demonstrate that information is public, one must be able to point to some fact that establishes that the information has become publicly available, such as the filing of a report with the SEC, the distribution of a press release, publishing the information on our website or posting on social media if those are regular ways we communicate with investors, or by other means that are reasonably designed to provide broad public access. Before a person with material nonpublic information can trade, the market must have adequate time to absorb the information that has been disclosed. For the purposes of this Insider Trading Policy, information will be considered public after the completion of one full day of trading following our public release of the information. For that purpose, a full day of trading means a session of regular trading hours Nasdaq Stock Market ("Nasdaq") between 9:30 a.m. and 4:00 p.m. Eastern Time (or such earlier closing time as has been set by exchange rules) has occurred.

For example, if the Company publicly discloses material nonpublic information of which you are aware before trading begins on a Tuesday, the first time you can buy or sell Company securities is the opening of the market on the following Wednesday. However, if the Company publicly discloses material information after trading begins on a Tuesday, the first time that you can buy or sell Company securities is the opening of the market on the following Thursday.

D. What are the Penalties for Insider Trading and Noncompliance with this Insider Trading Policy?

Both the SEC and the national securities exchanges, through the Financial Industry Regulatory Authority ("FINRA"), investigate and are very effective at detecting insider trading. The U.S. government pursues insider trading violations vigorously, successfully prosecuting, for example, trading by employees in foreign accounts, trading by family members and friends of insiders, and trading involving only a small number of shares.

The penalties for violating rules against insider trading can be severe and include:

- forfeiting any profit gained or loss avoided by the trading;
- payment of the loss suffered by the persons who, contemporaneously with the purchase or sale of securities that are subject of a violation, have purchased or sold securities of the same class;
- payment of criminal penalties of up to $5,000,000;
- payment of civil penalties of up to three times the profit made or loss avoided; and
- imprisonment for up to 20 years.

The Company and/or the supervisors of the person engaged in insider trading may also be required to pay civil penalties or fines of $2.5 million or more, up to three times the profit made or loss avoided, as well as criminal penalties of up to $25,000,000, and could under some circumstances be subject to private lawsuits.

Violation of this Insider Trading Policy or any federal or state insider trading laws may subject you to disciplinary action by the Company, including termination of your employment or other relationship with the Company. The Company reserves the right to determine, in its own discretion and on the basis of the information available to it, whether this Insider Trading Policy has been violated. The Company may determine that specific conduct violates this Insider Trading Policy whether or not it also violates the law. It is not necessary for the Company to await the filing or conclusion of a civil or criminal action against an alleged violator before taking disciplinary action.

E. How Do You Report a Violation of this Insider Trading Policy?

If you have a question about this Insider Trading Policy, including whether certain information you are aware of is material or has been made public, you should consult with the General Counsel. In addition, if you violate this Insider Trading Policy or any federal or state laws governing insider trading or know of any such violation by any director or employee of the Company, you should report the violation immediately to the General Counsel. However, if the conduct in question involves the General Counsel, or if you have reported such conduct to the General Counsel and you do not believe the General Counsel has dealt with it properly, or if you do not feel that you can discuss the matter with the General Counsel, you may raise the matter with the Chief Executive Officer.

PART II. TRADING PROCEDURES

A. Special Trading Restrictions Applicable to Insiders

In addition to needing to comply with the restrictions on trading in our securities set forth above, Insiders and their Affiliated Persons are subject to the following special trading restrictions:

1. Insider Trading Policy Modification.

The Company may at any time change this Insider Trading Policy or adopt such other policies or procedures which it considers appropriate to carry out the purposes of its policies regarding insider trading and the disclosure of Company information. Notice of any such change will be delivered to you by regular or electronic mail (or other delivery option used by the Company) by the Company. You will be deemed to have received, be bound by and agree to revisions of this Insider Trading Policy when such revisions have been delivered to you, unless you object to any revision in a written statement received by the General Counsel within two (2) business days of such delivery.

2. Prohibited Transactions

- ***No Short Sales***. You may not at any time sell any securities of the Company that are not owned by you at the time of the sale (a "short sale").

- ***No Purchases or Sales of Derivative Securities or Hedging Transactions***. You may not buy or sell puts, calls, other derivative securities of the Company or any derivative securities that provide the economic equivalent of ownership of any of the Company's securities or an opportunity, direct or indirect, to profit from any change in the value of our securities or engage in any other hedging transaction with respect to our securities.

- ***No Company Securities Subject to Margin Calls***. You may not use the Company's securities as collateral in a margin account.

- ***No Pledges***. You may not pledge Company securities as collateral for a loan (or modify an existing pledge).

3. Gifts and Other Distributions in Kind.

No Insider may donate or make any other transfer of Company securities without consideration when the Insider is not permitted to trade unless the donee agrees not to sell the shares until the Insider is permitted to sell. In addition to charitable donations or gifts to family members, friends, trusts or others, this prohibition applies to distributions to limited partners by limited partnerships that are subject to this Insider Trading Policy.

B. Pre-Clearance Procedures

No Insider may trade in our securities, even during an open trading window, unless the trade has been approved by the General Counsel in accordance with the procedures described below. In reviewing trading requests, the General Counsel may consult with our other officers and/or outside legal counsel and will seek approval of their own trades from the Chief Executive Officer.

1. Procedures. No Insider may trade in our securities unless:

- The Insider has notified the General Counsel of the nature of the proposed trade(s) using the then-current version of the Company's Stock Transaction Request form. If you are unsure about where to locate the current version of such document, please contact the General Counsel. To provide adequate time for the Company to process such request and, where applicable, to allow preparation of any required reports under Section 16 of the Exchange Act, a Stock Transaction Request form should be received by the General Counsel at least two (2) business days before the intended trade date;

- The Insider has certified to the General Counsel in writing before the proposed trade(s) that the Insider does not possess material nonpublic information concerning the Company;

- If the Insider is an executive officer or director, the Insider has informed the General Counsel of the amount of the proposed trade and whether, to the Insider's best knowledge, (a) the Insider has (or is deemed to have) engaged in any opposite way transactions within the previous six months that were not exempt from Section 16(b) of the Exchange Act and (b) if the transaction involves a sale by an "affiliate" of the Company or of "restricted securities" (as such terms are defined under Rule 144 under the Securities Act of 1933, as amended ("Rule 144")), whether the transaction meets all of the applicable conditions of Rule 144; and

- The General Counsel has approved the trade(s) and has certified their approval in writing (which may be by email).

The General Counsel does not assume responsibility for, and approval by the General Counsel does not protect the Insider from, the consequences of prohibited insider trading.

2. Additional Information.

Insiders shall provide to the General Counsel any documentation the General Counsel reasonably requires in furtherance of the foregoing procedures. Any failure to provide such information will be grounds for the General Counsel to deny approval of the trade request.

3. Notification of Brokers of Insider Status

Insiders who are required to file reports under Section 16 of the Exchange Act shall inform their broker-dealers that (a) the Insider is subject to Section 16; (b) the broker shall confirm that any trade by the Insider or any of their affiliates has been precleared by the Company; and (c) the broker is to provide transaction information to the Insider and/or General Counsel on the day of a trade.

4. No Obligation to Approve Trades.

The foregoing approval procedures do not in any way obligate the General Counsel to approve any trade. The General Counsel has sole discretion to reject any trading request.

From time to time, an event may occur that is material to the Company and is known by only by a limited number of directors and employees. The General Counsel may decline an Insider's request to preclear a proposed trade based on the existence of a material nonpublic development – even if the Insider is not aware of that material nonpublic development. If any Insider engages in a trade before a material nonpublic development is disclosed to the public or resolved, the Insider and the Company might be exposed to a charge of insider trading that could be costly and difficult to refute even if the Insider was unaware of the development. So long as the event remains material and nonpublic, the General Counsel may decide not to approve any transactions in the Company's securities. The General Counsel will subsequently notify the Insider once the material nonpublic development is disclosed to the public or resolved. If an Insider requests preclearance of a trade during the pendency of such an event, the General Counsel may reject the trading request without disclosing the reason.

5. Completion of Trades.

After receiving written clearance to engage in a trade signed by the General Counsel, an Insider must complete the proposed trade within three (3) business days or make a new trading request. Even if an Insider has received clearance, the Insider may not engage in a trade if (i) such clearance has been rescinded by the General Counsel, (ii) the Insider has otherwise received notice that the trading window has closed or (iii) the Insider has or acquires material nonpublic information.

6. Post-Trade Reporting.

The details of any transactions in our securities (including transactions effected pursuant to a Rule 10b5-1 Plan) by an Insider who is required to file reports under Section 16 of the Exchange Act must be reported to the General Counsel by the Insider or their brokerage firm on the same day on which a trade order is placed or such a transaction otherwise is entered into. The report shall include the date of the transaction, quantity of shares, the price and the name of the broker-dealer that effected the transaction. This reporting requirement may be satisfied by providing (or having the Insider's broker provide) a trade order confirmation to the General Counsel if the General Counsel receives such information by the required date. Compliance by directors and executive officers with this provision is imperative given the requirement of Section 16 of the Exchange Act that these persons generally report changes in ownership of Company securities within two (2) business days. The sanctions for noncompliance with this reporting deadline include mandatory disclosure in the Company's proxy statement for the next annual meeting of stockholders, as well as possible civil or criminal sanctions for chronic or egregious violators.

C. Exemptions

1. Pre-Approved Rule 10b5-1 Plan.

Transactions made pursuant to an approved Rule 10b5-1 Plan (as defined below) will not be subject to our trading windows or pre-clearance procedures, and Insiders are not required to complete a Stock Transaction Request form for such transactions. Rule 10b5-1 of the Exchange Act provides an affirmative defense from insider trading liability under the federal securities laws for trading plans, arrangements or instructions that meet specified requirements. A trading plan, arrangement or instruction that meets the requirements of the SEC's Rule 10b5-1 (a "Rule 10b5-1 Plan") enables Insiders to trade in Company securities outside of our trading windows, even when in possession of material nonpublic information.

If an Insider intends to trade pursuant to a Rule 10b5-1 Plan, such plan, arrangement or instruction must:

- satisfy the requirements of Rule 10b5-1;
- be documented in writing;
- be established during a trading window when such Insider does not possess material nonpublic information; and
- be pre-approved by the General Counsel.

Prior to approving a Rule 10b5-1 Plan, the General Counsel may require that the plan exclude or include certain provisions (e.g., cooling off period, minimum number of trades requirement, limited term) that ensure compliance with SEC regulations and practices the General Counsel deems to be in the best interests of the Company.

Any proposed deviation from the specifications of an approved Rule 10b5-1 Plan (including, without limitation, the amount, price or timing of a purchase or sale) must be reported immediately to, and be approved by, the General Counsel. **All transactions pursuant to a Rule 10b5-1 Plan must be timely reported in accordance with the procedures set forth above.**

Any modification or termination of a Rule 10b5-1 Plan previously approved by the General Counsel requires a new approval by the General Counsel. The General Counsel may require as a condition to such approval that the modification or termination occur during a trading window and that the Insider be not aware of material nonpublic information.

2. Employee Equity and Retirement Plans.

Exercise of Stock Options. The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to the exercise for cash of an option to purchase securities of the Company. However, the exercise is subject to the current reporting requirements of Section 16 of the Exchange Act and, therefore, Insiders must comply with the post-trade reporting requirement described in Section C above for any such transaction. In addition, the securities acquired upon the exercise of an option to purchase Company securities are subject to all of the requirements of this Insider Trading

Policy, including the Trading Procedures. Moreover, the Trading Procedures apply to the use of outstanding Company securities to pay part or all of the exercise price of an option, any net option exercise, any exercise of a stock appreciation right, share withholding and any sale of stock as part of a broker-assisted cashless exercise of an option or any other market sale for the purpose of generating the cash needed to pay the exercise price of an option.

Tax Withholding on Restricted Stock/Units. The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to the withholding by the Company of shares of stock upon vesting of restricted stock or upon settlement of restricted stock units to satisfy tax withholding requirements if (a) withholding is required by the applicable plan or award agreement or (b) the election to exercise the tax withholding right was made by the Insider in compliance with the Trading Procedures.

Employee Stock Purchase Plan. The trading prohibitions and restrictions set forth in the Trading Procedures do not apply to periodic wage withholding contributions by the Company or its employees that are used to purchase Company stock pursuant to the employees' advance instructions under the Company's Employee Stock Purchase Plan. However, an Insider may not: (a) elect to participate in the plan or alter their instructions regarding the level of withholding or purchase by the Insider of Company securities under the plan; or (b) make cash contributions to the plan (other than through periodic wage withholding) without complying with the Trading Procedures. Any sale of securities acquired under the plan is subject to the prohibitions and restrictions of the Trading Procedures.

D. Waivers

A waiver of any provision of this Insider Trading Policy or the Trading Procedures may be authorized in writing by the General Counsel. All waivers shall be reported to the Board of Directors.

PART III. ACKNOWLEDGEMENT

We will deliver a copy of this Insider Trading Policy to all current employees and directors and consultants and to future employees and directors and consultants at the start of their employment or relationship with the Company. Each of these individuals must acknowledge that they have received a copy and agree to comply with the terms of this Insider Trading Policy, and, if applicable, the Trading Procedures contained herein. The attached acknowledgment must be completed and submitted to the Company within ten days of receipt.

At our request, directors and employees and consultants will be required to re-acknowledge and agree to comply with the Insider Trading Policy (including any amendments or modifications). For that purpose, an individual will be deemed to have acknowledged and agreed to comply with the Insider Trading Policy, as amended from time to time, when copies of those items have been delivered by regular or electronic mail (or other delivery option used by the Company) to the General Counsel.

* * *

Questions regarding this Insider Trading Policy are encouraged and may be directed to the General Counsel.

ADOPTED: December 29, 2022.

AS AMENDED ON: September 17, 2024

EXHIBIT A

ACKNOWLEDGEMENT

I hereby acknowledge that I have read, that I understand, and that I agree to comply with the Insider Trading Policy of Disc Medicine, Inc. (the "Company"). I further acknowledge and agree that I am responsible for ensuring compliance with the Insider Trading Policy and the Trading Procedures by all of my "Affiliated Persons." I also understand and agree that I will be subject to sanctions, including termination of employment, that may be imposed by the Company, in its sole discretion, for violation of the Insider Trading Policy, and that the Company may give stop-transfer and other instructions to the Company's transfer agent or any brokerage firm managing the Company's equity incentive plan(s) against the transfer of any Company securities that the Company considers to be in contravention of the Insider Trading Policy.

This acknowledgement constitutes consent for the Company to impose sanctions for violation of the Insider Trading Policy, including the Trading Procedures, and to issue any stop-transfer orders to the Company's transfer agent that the Company, in its sole discretion, deems appropriate to ensure compliance.

Date: _____ Signature: _____

Name: _____

Title: _____

Send signed Acknowledgement to:

[Name]
[Title]
[Company Name]
[Address]

Exhibit 21.1

Legal Name	State of Organization
Disc Medicine Opco, Inc.	Delaware
Disc Medicine Securities Corp.	Massachusetts

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-3 No. 333-269270) of Disc Medicine, Inc.,

(2) Registration Statement (Form S-3 No. 333-281359) of Disc Medicine, Inc.,

(3) Registration Statement (Form S-8 No. 333-278129) pertaining to the Amended and Restated 2021 Stock Option and Incentive Plan, Amended and Restated 2021 Employee Stock Purchase Plan, the Stock Option Inducement Awards, and the Restricted Stock Unit Inducement Awards of Disc Medicine, Inc.,

(4) Registration Statement (Form S-8 No. 333-269271) pertaining to the 2017 Stock Option and Grant Plan, Amended and Restated 2021 Stock Option and Incentive Plan, and Amended and Restated 2021 Employee Stock Purchase Plan of Disc Medicine, Inc.

(5) Registration Statement (Form S-8 No. 333-263410) pertaining to the 2021 Stock Option and Incentive Plan of Gemini Therapeutics, Inc.,

(6) Registration Statement (Form S-8 No. 333-255194) pertaining to the 2021 Stock Option and Incentive Plan and the 2021 Inducement Plan of Gemini Therapeutics, Inc., and

(7) Registration Statement (Form S-8 No. 333-260243) pertaining to the 2021 Employee Stock Purchase Plan of Gemini Therapeutics, Inc.;

of our reports dated February 27, 2025, with respect to the consolidated financial statements of Disc Medicine, Inc. and the effectiveness of internal control over financial reporting of Disc Medicine, Inc. included in this Annual Report (Form 10-K) of Disc Medicine, Inc. for the year ended December 31, 2024.

/s/ Ernst & Young LLP

Boston, Massachusetts
February 27, 2025

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO RULE 13A-14(A) / RULE 15D-14(A) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, John Quisel, J.D., Ph.D., certify that:

1. I have reviewed this Annual Report on Form 10-K of Disc Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 27, 2025

/s/ John Quisel

John Quisel, J.D., Ph.D.
President, Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO RULE 13A-14(A) / RULE 15D-14(A) OF THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Jean Franchi, certify that:

1. I have reviewed this Annual Report on Form 10-K of Disc Medicine, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 27, 2025

/s/ Jean Franchi

Jean Franchi
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATIONS OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Annual Report on Form 10-K of Disc Medicine, Inc. (the "Company") for the fiscal year ended December 31, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his or her knowledge:

1. the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: February 27, 2025

/s/ John Quisel

John Quisel, J.D., Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

/s/ Jean Franchi

Jean Franchi
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 97.1

DISC MEDICINE, INC.

COMPENSATION RECOVERY POLICY

Adopted September 19, 2023

As amended on September 17, 2024

Disc Medicine, Inc., a Delaware corporation (the "Company"), has adopted a Compensation Recovery Policy (this "Policy") as described below.

1. Overview

The Policy sets forth the circumstances and procedures under which the Company shall recover Erroneously Awarded Compensation from Covered Persons (as defined below) in accordance with rules issued by the United States Securities and Exchange Commission (the "SEC") under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and the Nasdaq Stock Market. Capitalized terms used and not otherwise defined herein shall have the meanings given in Section 3 below.

2. Compensation Recovery Requirement

In the event the Company is required to prepare a Financial Restatement, the Company shall recover reasonably promptly all Erroneously Awarded Compensation with respect to such Financial Restatement.

3. Definitions

a. "Applicable Recovery Period" means the three completed fiscal years immediately preceding the Restatement Date for a Financial Restatement. In addition, in the event the Company has changed its fiscal year: (i) any transition period of less than nine months occurring within or immediately following such three completed fiscal years shall also be part of such Applicable Recovery Period and (ii) any transition period of nine to 12 months will be deemed to be a completed fiscal year.

b. "Applicable Rules" means any rules or regulations adopted by the Exchange pursuant to Rule 10D-1 under the Exchange Act and any applicable rules or regulations adopted by the SEC pursuant to Section 10D of the Exchange Act.

c. "Board" means the Board of Directors of the Company.

d. "Committee" means the Compensation Committee of the Board or, in the absence of such committee, a majority of independent directors serving on the Board.

e. "Covered Person" means any Executive Officer and any other person designated by the Board or the Committee as being subject to this Policy. A person's status as a Covered Person with respect to Erroneously Awarded Compensation shall be determined as of the time of receipt of such Erroneously Awarded Compensation

regardless of the person's current role or status with the Company (e.g., if a person began service as an Executive Officer after the beginning of an Applicable Recovery Period, that person would not be considered a Covered Person with respect to Erroneously Awarded Compensation received before the person began service as an Executive Officer, but would be considered a Covered Person with respect to Erroneously Awarded Compensation received after the person began service as an Executive Officer where such person served as an Executive Officer at any time during the performance period for such Erroneously Awarded Compensation).

f. "Effective Date" means October 2, 2023.

g. "Erroneously Awarded Compensation" means the amount of any Incentive-Based Compensation received by a Covered Person on or after the Effective Date and during the Applicable Recovery Period that exceeds the amount that otherwise would have been received by the Covered Person had such compensation been determined based on the restated amounts in a Financial Restatement, computed without regard to any taxes paid. Calculation of Erroneously Awarded Compensation with respect to Incentive-Based Compensation based on stock price or total shareholder return, where the amount of Erroneously Awarded Compensation is not subject to mathematical recalculation directly from the information in a Financial Restatement, shall be based on a reasonable estimate of the effect of the Financial Restatement on the stock price or total shareholder return upon which the Incentive-Based Compensation was received, and the Company shall maintain documentation of the determination of such reasonable estimate and provide such documentation to the Exchange in accordance with the Applicable Rules. Incentive-Based Compensation is deemed received, earned, or vested when the Financial Reporting Measure is attained, not when the actual payment, grant, or vesting occurs.

h. "Exchange" means the Nasdaq Stock Market LLC.

i. An "Executive Officer" means any person who served the Company in any of the following roles at any time during the performance period applicable to Incentive-Based Compensation such person received during service in such role: the president, principal financial officer, principal accounting officer (or if there is no such accounting officer the controller), any vice president in charge of a principal business unit, division, or function (such as sales, administration, or finance), any other officer who performs a policy making function, or any other person who performs similar policy making functions for the Company. Executive officers of parents or subsidiaries of the Company may be deemed executive officers of the Company if they perform such policy making functions for the Company.

j. "Financial Reporting Measures" mean measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, any measures that are derived wholly or in part from such measures (including, for example, a non-GAAP financial measure), and stock price and total shareholder return.

k. "Incentive-Based Compensation" means any compensation provided, directly or indirectly, by the Company or any of its subsidiaries that is granted, earned, or vested based, in whole or in part, upon the attainment of a Financial Reporting Measure and any other equity-based compensation provided by the Company or any of its subsidiaries, including, without limitation, stock options, restricted stock awards, restricted stock units and stock appreciation rights. For avoidance of doubt, Incentive-Based Compensation is "received" for purposes of this Policy in the fiscal period during which the Financial Reporting Measure specified in the Incentive-Based Compensation award is attained, even if the payment or grant of such Incentive-Based Compensation occurs after the end of that period.

l. A "Financial Restatement" means a restatement of previously issued financial statements of the Company due to the material noncompliance of the Company with any financial reporting requirement under the securities laws, including any required restatement to correct an error in previously-issued financial statements that is material to the previously-issued financial statements or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period.

m. "Restatement Date" means, with respect to a Financial Restatement, the earlier to occur of: (i) the date the Board or the Audit Committee of the Board concludes, or reasonably should have concluded, that the Company is required to prepare the Financial Restatement or (ii) the date a court, regulator or other legally authorized body directs the Company to prepare the Financial Restatement.

4. Exception to Compensation Recovery Requirement

The Company may elect not to recover Erroneously Awarded Compensation pursuant to this Policy if the Committee determines that recovery would be impracticable, and one or more of the following conditions, together with any further requirements set forth in the Applicable Rules, are met: (i) the direct expense paid to a third party, including outside legal counsel, to assist in enforcing this Policy would exceed the amount to be recovered, and the Company has made a reasonable attempt to recover such Erroneously Awarded Compensation; or (ii) recovery would likely cause an otherwise tax-qualified retirement plan to fail to be so qualified under applicable regulations.

5. Recovery from Participating Employees

In addition to (and without limiting) the provisions of paragraph 2 above, in the event the Company is required to prepare a Financial Restatement after the Effective Date, the Company may recover from any current or former employee of the Company who is not a Covered Person (each a "Participating Employee") and who received Incentive-Based Compensation from the Company during the three completed fiscal years immediately preceding the date on which the Board or the Audit Committee determines that the Company is required to prepare a Financial Restatement, the amount that exceeds what would have been paid to the Participating Employee under the Financial Restatement; provided that, this paragraph 5 will apply only to the extent the

Board (or a duly established committee thereof), in its sole discretion, determines that the Participating Employee committed any act or omission that materially contributed to the circumstances requiring the Financial Restatement and such act or omission involved any of the following: (i) misconduct, wrongdoing or a violation of any of the Company's rules or of any applicable legal or regulatory requirements in the course of the Participating Employee's employment by the Company; or (ii) a breach of a fiduciary duty to the Company or its stockholders by the Participating Employee.

6. Recovery Where Intentional Misconduct

In addition to (and without limiting) the provisions of paragraph 2 and 5 above, in the event the Company is required to prepare a Financial Restatement after the Effective Date and the Board (or a duly established committee thereof), in its sole discretion, determines that a Covered Person's or a Participating Employee's act or omission contributed to the circumstances requiring the Financial Restatement and such act or omission involved any of the following: (i) willful, knowing or intentional misconduct or a willful, knowing or intentional violation of any of the Company's rules or any applicable legal or regulatory requirements in the course of the Covered Person's or the Participating Employee's employment by the Company or (ii) fraud in the course of the Covered Person's or the Participating Employee's employment by the Company, the Company may recover from such Covered Person or Participating Employee up to 100% (as determined by the Board or a duly established committee thereof in its sole discretion) of the Incentive-Based Compensation received by such Covered Person or Participating Employee from the Company during the three fiscal years preceding the date on which the Company determined that it is required to prepare a Financial Restatement.

7. Tax Considerations

To the extent that, pursuant to this Policy, the Company is entitled to recover any Erroneously Awarded Compensation that is received by a Covered Person, the gross amount received (i.e., the amount the Covered Person received, or was entitled to receive, before any deductions for tax withholding or other payments) shall be returned by the Covered Person.

8. Method of Compensation Recovery

The Committee shall determine, in its sole discretion, the method for recovering Erroneously Awarded Compensation hereunder, which may include, without limitation, any one or more of the following:

a. requiring reimbursement of cash Incentive-Based Compensation previously paid;

b. seeking recovery of any gain realized on the vesting, exercise, settlement, sale, transfer or other disposition of any equity-based awards;

c. cancelling or rescinding some or all outstanding vested or unvested equity-based awards;

d. adjusting or withholding from unpaid compensation or other set-off;

e. cancelling or offsetting against planned future grants of equity-based awards; and/or

f. any other method permitted by applicable law or contract.

The Committee need not utilize the same method of recovery from all Covered Persons or with respect to all types of Erroneously Awarded Compensation.

A Covered Person will be deemed to have satisfied such person's obligation to return Erroneously Awarded Compensation to the Company if such Erroneously Awarded Compensation is returned in the exact same form in which it was received; provided that equity withheld to satisfy tax obligations will be deemed to have been received in cash in an amount equal to the tax withholding payment made.

In the event the Company is required to recover Erroneously Awarded Compensation from a Covered Person who is no longer an employee, the Company is entitled to seek such recovery in order to comply with applicable law, regardless of the terms of any release of claims or separation agreement such individual may have signed.

9. Policy Interpretation

This Policy shall be interpreted in a manner that is consistent with the Applicable Rules and any other applicable law. The Committee shall take into consideration any applicable interpretations and guidance of the SEC in interpreting this Policy, including, for example, in determining whether a financial restatement qualifies as a Financial Restatement hereunder. To the extent the Applicable Rules require recovery of Incentive-Based Compensation in additional circumstances besides those specified above, nothing in this Policy shall be deemed to limit or restrict the right or obligation of the Company to recover Incentive-Based Compensation to the fullest extent required by the Applicable Rules.

10. Policy Administration

This Policy shall be administered by the Committee; provided, however, that the Board shall have exclusive authority to authorize the Company to prepare a Financial Restatement. In doing so, the Board may rely on a recommendation of the Audit Committee of the Board. The Committee shall have such powers and authorities related to the administration of this Policy as are consistent with the governing documents of the Company and applicable law. The Committee shall have full power and authority to take, or direct the taking of, all actions and to make all determinations required or provided for under this Policy and shall have full power and authority to take, or direct the taking of, all such other actions and make all such other determinations not inconsistent with the specific terms and provisions of this Policy that the Committee deems to be necessary or appropriate to the administration of this Policy. The interpretation and construction by the Committee of any provision of this Policy and all determinations made by the Committee under this policy shall be final, binding and conclusive.

11. Compensation Recovery Repayments not Subject to Indemnification

Notwithstanding anything to the contrary set forth in any agreement with, or the organizational documents of, the Company or any of its subsidiaries, Covered Persons are not entitled to indemnification for Erroneously Awarded Compensation or for any claim or losses arising out of or in any way related to Erroneously Awarded Compensation recovered under this Policy.

12. No Impairment of Other Remedies

Nothing contained in this Policy, and no recoupment or recovery as contemplated herein, shall limit any claims, damages or other legal remedies the Company or any of its affiliates may have against a Covered Person arising out of or resulting from any actions or omissions by the Covered Person. This Policy does not preclude the Company from taking any other action to enforce a Covered Person's obligations to the Company, including, without limitation, termination of employment and/or institution of civil proceedings. This Policy is in addition to the requirements of Section 304 of the Sarbanes-Oxley Act of 2002 ("SOX 304") that are applicable to the Company's Chief Executive Officer and Chief Financial Officer and to any other compensation recoupment policy and/or similar provisions in any employment, equity plan, equity award, or other individual agreement, to which the Company is a party or which the Company has adopted or may adopt and maintain from time to time; provided, however, that compensation recouped pursuant to this Policy shall not be duplicative of compensation recouped pursuant to SOX 304 or any such compensation recoupment policy and/or similar provisions in any such employment, equity plan, equity award, or other individual agreement except as may be required by law.

13. Recovery Requirement Shall not Constitute "Good Reason" Under Employment or Other Compensation Agreements

Any action by the Company to recoup or any recoupment of Erroneously Awarded Compensation under this Policy from a Covered Person shall not be deemed (i) "good reason" for such Covered Person's resignation or to serve as a basis for a claim of constructive termination under any employment or severance agreement with the Company or under the terms of any benefits or compensation arrangement applicable to such Covered Person, or (ii) to constitute a breach of a contract or other arrangement to which such Covered Person is party.

14. Amendment; Termination

The Committee may amend this Policy in its discretion, including as it deems necessary to comply with the regulations adopted by the SEC under Rule 10D-1 and the rules of any national securities exchange or national securities association on which the Company's securities are listed. The Committee may terminate this Policy at any time. Notwithstanding anything herein to the contrary, no amendment or termination of this Policy shall be effective if that amendment or termination would cause the Company to violate any federal securities laws, SEC rules or the

rules of any national securities exchange or national securities association on which the Company's securities are listed.

15. Successors

This Policy shall be binding and enforceable against all Covered Executives and their successors, beneficiaries, heirs, executors, administrators, or other legal representatives.

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ACKNOWLEDGMENT

(to be signed by all Covered Persons)

I, the undersigned, agree and acknowledge that I am fully bound by, and subject to, all of the terms and conditions of the Disc Medicine, Inc. Compensation Recovery Policy (as may be amended, restated, supplemented or otherwise modified from time to time, the "Policy") and that I have been provided a copy of the Policy. In the event of any inconsistency between the Policy and the terms of any employment or similar agreement to which I am a party, or the terms of any compensation plan, program or agreement under which any compensation has been granted, awarded, earned or paid, the terms of the Policy shall govern. If the Committee determines that any amounts granted, awarded, earned or paid to me must be forfeited or reimbursed to the Company, I will promptly take any action necessary to effectuate such forfeiture and/or reimbursement.

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Name: