



Disc Medicine Provides Update on Hematology Portfolio and Outlines Near-Term Business Objectives and Anticipated Milestones

October 20, 2025

- Submitted a New Drug Application (NDA) for accelerated approval of bitopertin in erythropoietic protoporphyria (EPP) on September 29, 2025, and subsequently was awarded the FDA Commissioner's National Priority Voucher (CNPV), a program designed to shorten the expected NDA review period to 1-2 months
- Acceleration of ongoing efforts to support the potential earlier approval and commercialization of bitopertin for EPP in the US, expected in late 2025 or early 2026
- Plans to advance DISC-0974 for anemia of myelofibrosis (MF) and DISC-3405 for polycythemia vera (PV) deeper into development and to continue exploring potential role of hepcidin in additional indications
- Strong financial position ending Q3 with approximately \$616 million in cash, cash equivalents, and marketable securities

WATERTOWN, Mass., Oct. 20, 2025 (GLOBE NEWSWIRE) -- Disc Medicine, Inc. (NASDAQ:IRON), a clinical-stage biopharmaceutical company focused on the discovery, development, and commercialization of novel treatments for patients suffering from serious hematologic diseases, today outlined its recent pipeline and operational progress and strategic priorities for the rest of 2025 and 2026.

"2025 has been another eventful year for Disc Medicine, and we are accelerating development across three key indications—EPP and our myeloproliferative neoplasm portfolio, MF and PV. For bitopertin, with the recent grant of the FDA's CNPV, our focus is on accelerating our commercial readiness to ensure access to patients as quickly as possible upon approval, if granted," said John Quisel, J.D., Ph.D., Chief Executive Officer and President of Disc Medicine.

"We're also excited about the progress across our iron homeostasis portfolio, DISC-0974 and DISC-3405, as these programs will be important drivers of Disc's future growth. We're positioning DISC-0974 for development in anemia of MF and look forward to providing an initial set of data from the Phase 2 RALLY-MF trial of DISC-0974 in its lead indication, anemia of MF, by the end of this year. Additionally, we are advancing the development of DISC-3405 in PV with data expected next year. We also continue to drive our expansion strategy for both of these programs with a plan to initiate a Phase 2 trial of DISC-0974 in anemia of inflammatory bowel disease in 2026 and a Phase 1b trial of DISC-3405 in sickle cell disease by year end."

Key Near-Term Business Objectives

Bitopertin: GlyT1 Inhibitor (Heme Synthesis Modulator)

- Accelerate activities to support a potential US approval and launch in late 2025 or early 2026 based on accelerated review timeline associated with receipt of the CNPV, which is designed to shorten the NDA review process to 1-2 months
- Drive enrollment of ongoing APOLLO confirmatory trial of bitopertin in EPP that is intended to also support potential approval of bitopertin in territories outside the US

DISC-0974: Anti-hemojuvelin Antibody (Hepcidin Suppression)

- Progress and position DISC-0974 to be advanced into potential late-stage clinical development for anemia of MF
- Initial data from ongoing Phase 2 study in anemia of MF to be reported by year end; topline data expected in 2026, if positive, is anticipated to support discussion with regulatory agencies on registrational path
- Demonstrate role of hepcidin suppression and anti-hemojuvelin (HJV) mechanism in other anemias of chronic disease:
 - Recently completed Phase 1b double-blind, placebo-controlled study of DISC-0974 demonstrated engagement of mechanism in anemia of non-dialysis dependent chronic kidney disease (NDD-CKD) with variable effects on hemoglobin
 - DISC-0974 was generally well-tolerated with substantial decreases in hepcidin, increases in iron, and improvements in markers of erythropoiesis
 - Meaningful hemoglobin increases observed in only a subset of patients were in part driven by those with higher baseline erythropoietin (EPO) levels
 - Full data will be shared at the upcoming 2025 ASN Kidney Week; we are assessing options for the program based on full analysis of the data
 - Initiation of a Phase 2 study in patients with IBD and anemia anticipated in Q1 2026, and planning exploratory studies in additional patient populations with anemia of chronic disease.
- Plans to accelerate next-generation, long-acting anti-HJV antibody into IND-enabling studies

DISC-3405: Anti-TMPRSS6 Antibody (Hepcidin Induction)

- Establish Phase 2 proof-of-concept of DISC-3405 for PV and support potential development into later stage development

- Topline data from ongoing Phase 2 study in PV expected in 2026
- Initiation of Phase 1b study in patients with SCD anticipated by year end
- Plans to explore the role of therapeutic iron restriction in other indications

Bitopertin, DISC-0974, and DISC-3405 are investigational agents and are not approved for use as therapies in any jurisdiction worldwide.

About Disc Medicine

Disc Medicine (NASDAQ:IRON) is a clinical-stage biopharmaceutical company committed to discovering, developing, and commercializing novel treatments for patients who suffer from serious hematologic diseases. We are building a portfolio of innovative, potentially first-in-class therapeutic candidates that aim to address a wide spectrum of hematologic diseases by targeting fundamental biological pathways of red blood cell biology, specifically heme biosynthesis and iron homeostasis. For more information, please visit www.discmedicine.com.

Disc Cautionary Statement Regarding Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, express or implied statements regarding Disc’s expectations with respect to the next stages of its development programs for bitopertin, DISC-0974 and DISC-3405, including projected timelines for the initiation and completion of its clinical trials, anticipated timing of release of data, and other clinical activities; the registrational pathway for bitopertin, including the potential for accelerated approval, benefits of the CNPV and expected review period; anticipated discussions with regulatory agencies; ongoing preparations for the potential launch of bitopertin; the potential of its development programs in new indications; and the strength of its financial position and its anticipated cash runway. The use of words such as, but not limited to, “believe,” “expect,” “estimate,” “project,” “intend,” “future,” “potential,” “continue,” “may,” “might,” “plan,” “will,” “should,” “seek,” “anticipate,” or “could” or the negative of these terms and other similar words or expressions that are intended to identify forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on Disc’s current beliefs, expectations and assumptions regarding the future of Disc’s business, future plans and strategies, clinical results and other future conditions. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

Disc may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and investors should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements as a result of a number of material risks and uncertainties including but not limited to: the adequacy of Disc’s capital to support its future operations and its ability to successfully initiate and complete clinical trials; the nature, strategy and focus of Disc; the difficulty in predicting the time and cost of development of Disc’s product candidates; Disc’s plans to research, develop and commercialize its current and future product candidates; the timing of initiation of Disc’s planned preclinical studies and clinical trials; the timing of the availability of data from Disc’s clinical trials; Disc’s ability to identify additional product candidates with significant commercial potential and to expand its pipeline in hematological diseases; the timing and anticipated results of Disc’s preclinical studies and clinical trials and the risk that the results of Disc’s preclinical studies and clinical trials may not be predictive of future results in connection with future studies or clinical trials and may not support further development and marketing approval; the content and timing of decisions made by the FDA and other regulatory authorities; and the other risks and uncertainties described in Disc’s filings with the Securities and Exchange Commission, including in the “Risk Factors” section of Disc’s Annual Report on Form 10-K for the year ended December 31, 2024, and in subsequent Quarterly Reports on Form 10-Q. Any forward-looking statement speaks only as of the date on which it was made. None of Disc, nor its affiliates, advisors or representatives, undertake any obligation to publicly update or revise any forward-looking statement, whether as result of new information, future events or otherwise, except as required by law.

Media Contact

Peg Rusconi
Deerfield Group
peg.rusconi@deerfieldgroup.com

Investor Relations Contact

Christina Tartaglia
Precision AQ
christina.tartaglia@precisionaq.com