Disc Medicine Highlights Recent Achievements Across Hematology Portfolio and Key Business Objectives and Milestones for 2025

January 10, 2025

- *In Q1 2025, the Company will discuss the plans for NDA submission under a potential accelerated approval path for bitopertin in erythropoietic protoporphyria (EPP), following a Type C meeting with FDA on confirmatory study design*
- *Initial data from an ongoing phase 2 study of DISC-0974 (anti-hemojuvelin antibody) in anemia of myelofibrosis (MF) expected H2 2025*
- *Data from higher doses and multiple dose cohorts of a phase 1b study of DISC-0974 in anemia of chronic kidney disease (CKD) expected H2 2025*
- *Initiation of phase 2 study of DISC-3405 (anti-TMPRSS6 antibody) in polycythemia vera (PV) planned for H1 2025*

WATERTOWN, Mass., Jan. 10, 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 2025.

“This past year has been another one of tremendous execution and achievement for Disc. During 2024, we successfully advanced every clinical program in our portfolio, transformed our company into a late-stage development organization, and fortified our balance sheet to position us well for Disc’s next chapter,” said John Quisel, J.D., Ph.D., Chief Executive Officer and President of Disc. “Most prominently, we completed and presented positive results from our two phase 2 studies of bitopertin in EPP, which showed that reducing PPIX in the blood results in marked improvements across multiple clinical outcomes in EPP patients. This culminated in a successful end-of-phase 2 meeting with the FDA, which provided a path for potential accelerated approval. We will provide an update this quarter on our NDA plans following discussion with the FDA on the design of APOLLO, our planned confirmatory study.

We’re equally excited about the data we presented this year from our iron homeostasis portfolio, DISC-0974 and DISC-3405, as these programs will be important drivers of Disc’s future growth. In particular, we were encouraged by the robust results from our phase 1b study of DISC-0974 for anemia of myelofibrosis. These data were recently shared in an oral presentation at the 66th American Society of Hematology Annual Meeting and suggest that DISC-0974 has the potential for best-in-class activity. We’ve now begun the phase 2 portion of this study and look forward to presenting the initial findings later this year.”

Summary of Key Achievements During 2024

- Positive end-of-phase 2 meeting with FDA providing a path toward potential accelerated approval for bitopertin in EPP
- Presentation of full results from phase 2 AURORA and BEACON studies demonstrating significant reductions in PPIX are associated with substantial improvements in time spent in sunlight, measures of quality of life, and reduction in phototoxic reactions
- Presentation of positive data from the phase 1b study of DISC-0974 (anti-hemojuvelin antibody) for anemia of myelofibrosis (MF), demonstrating robust and broad hematologic activity across patient segments
    - Results showed substantial and durable improvements in hemoglobin, reductions in transfusion burden, and improvements in fatigue scores
    - Initiated the phase 2 portion of the study after positive discussions with FDA
- Presentation of data from initial cohorts of the ongoing phase 1b study of DISC-0974 in anemia of non-dialysis dependent CKD patients, demonstrating hematologic activity following a single dose
- Presentation of positive, first-in-human data from the phase 1 SAD / MAD study of DISC-3405 (anti-TMPRSS6 antibody) in healthy volunteers, demonstrating proof-of-mechanism with substantial, dose-dependent increases in hepcidin and reductions in serum iron supportive of a once-monthly dosing regimen
- Strengthened balance sheet through an equity offering with gross proceeds of approximately $178 million and a non-dilutive debt facility, which provide cash runway well into 2027, and expanded executive team to support next phase of company’s growth

Key Business Objectives and Milestones for 2025

Bitopertin: GlyT1 Inhibitor (Heme Synthesis Modulator)

Pursue development and global registration of bitopertin in erythropoietic protoporphyria (EPP), with the potential for accelerated approval in the U.S. using PPIX as surrogate endpoint

- Provide update in Q1 2025 on design of confirmatory APOLLO trial and plans for NDA submission, following Type C

meeting with FDA
- Initiate global, confirmatory APOLLO trial by mid-2025
- Obtain protocol assistance and feedback on regulatory path from EMA
- Continue to advance pre-commercialization and launch preparation activities

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

Pursue next stage of development of DISC-0974 for the treatment of anemia in myelofibrosis patients and generate additional data supporting activity and dose selection in CKD anemia

- Progress ongoing phase 2 MF anemia trial with initial data expected H2 2025
- Progress ongoing phase 1b CKD anemia trial with multiple-dose data expected H2 2025
- Continue to develop scientific rationale for hepcidin suppression in other anemias

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

Initiate phase 2 clinical trial of DISC-3405 in PV and broaden role of iron restriction in other indications

- Plan to initiate phase 2 clinical trial of DISC-3405 in PV in H1 2025
- Continued to develop scientific rationale for therapeutic iron restriction in sickle cell disease and 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; 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; and the other risks and uncertainties described in Disc's filings with the Securities and Exchange Commission, including in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2023, 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