Disc Presents Positive Updated Results from Phase 2 BEACON Study of Bitopertin and Other Programs at the 65th American Society of Hematology (ASH) Annual Meeting

December 12, 2023

- *Updated data from BEACON continued to demonstrate significant, consistent reductions in protoporphyrin IX (PPIX) > 40% and improvements in sunlight tolerance*
- *Robust and consistent improvements across all measures of sunlight tolerance, including >3x improvement over historical control of precedented pivotal endpoint*
- *Bitopertin was generally well-tolerated with stable hemoglobin at both dose levels*
- *Earlier today, Disc also announced initial positive data from the phase 1b study of DISC-0974 in myelofibrosis patients with anemia, demonstrating improvements in hemoglobin and reductions in transfusion burden*

WATERTOWN, Mass., Dec. 11, 2023 (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 presented updated results from the phase 2 open-label BEACON study of bitopertin, an orally administered glycine transporter 1 (GlyT1) inhibitor, in patients with erythropoietic protoporphyria (EPP) as an oral presentation at the 65th ASH Annual Meeting. The updated data are consistent with and confirm the initial positive results presented in June, demonstrating significant decreases in PPIX, robust and consistent improvements in sunlight tolerance across all study measures, including the precedented pivotal endpoint, and improvements in patient quality of life.

“This has been a tremendous ASH meeting for Disc, as we presented data across our two most advanced programs. We are especially proud and excited to present the updated results from BEACON, which reflect a more robust open label data set and clearly indicate that reducing PPIX with bitopertin has the potential to result in dramatic benefits for patients with EPP. Importantly, this improvement was observed across every efficacy measure of the study, including our analysis of the precedented pivotal endpoint, cumulative time in light over 6 months, which we debuted at this meeting,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer. “With these results and the positive initial efficacy data from the DISC-0974 myelofibrosis study that we presented earlier today, Disc is preparing to enter its next stage of growth. We look forward to next year as we advance our full portfolio and obtain the readouts from AURORA and other studies.”

The BEACON study (ACTRN12622000799752) is a randomized, open-label, parallel-arm study that enrolled 22 adult subjects with EPP or X-linked protoporphyria (XLP) in Australia, and has been expanded to include adolescents. This trial was designed to assess changes in levels of PPIX, as well as measures of photosensitivity, quality of life, and safety and tolerability. Subjects are randomized to receive either 20 mg or 60 mg of bitopertin once-daily for 24 weeks, after which patients have the option of continuing in an open-label extension of the trial for up to an additional 24 weeks. The updated data presented reflects results from all 22 adults, with a data cutoff of September 18, 2023 for PPIX data and October 20, 2023 for all other endpoints. The data are consistent with and confirm the initial positive results presented in June 2023.

- Protoporphyrin IX (PPIX) levels: Significant, dose-dependent, and sustained reductions in whole blood PPIX levels; mean reduction > 40% ($p<0.001$ versus baseline)
- Demonstrated substantial and consistent improvements in sunlight tolerance across all study measures
- Highlights of the data presented:
    - Average time to prodrome: Greater than 3x improvement vs. baseline ($p<0.001$)
    - Increased proportion of days without symptoms: 78% vs. 33% (baseline)
    - Increased proportion of sunlight challenges without prodromes: 54% vs. 7% (baseline)
    - Phototoxic reactions: 92% reduction in patient-reported reactions while on treatment compared to baseline
    - Nearly all participants reported improvements in multiple quality-of-life measures at the end of study
- Mean cumulative total time in light on days without pain observed over the 6-month treatment period (precedented pivotal endpoint): 222.6 $\pm$ 129.3 hours
    - Bitopertin-treated participants had a >3x increase relative to historical control
- Bitopertin was generally well tolerated at both dose levels with no serious adverse events, stable mean hemoglobin levels, and no anemia adverse events (AEs) reported.
    - The most common AEs were dizziness, lightheadedness, headache, and nausea.

Earlier today, Disc also presented initial positive data from the ongoing phase 1b study of DISC-0974 in myelofibrosis (MF) patients with anemia. The data were presented as a poster during the ASH meeting and demonstrated suppression of hepcidin, increased iron levels and improvements in hematologic parameters, including increased hemoglobin and reduction in transfusion burden. The presentation was announced in a separate press release issued earlier today and will be reviewed again during the management call, as well as initial data from the first dose-escalation cohort of the ongoing phase 1b/2 study in non-dialysis dependent chronic kidney disease (NDD-CKD) patients with anemia.

Bitopertin and DISC-0974 are investigational agents and are not approved for use as a therapy in any jurisdiction worldwide.

Webcast Conference Call Information
Management will host a call on Monday, December 11th at 9:30 pm ET / 6:30 pm PT to review data and operational plans. Please register for management's webcast on the Events and Presentations page of Disc's website (https://ir.discmedicine.com/).

About Disc Medicine
Disc Medicine 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 Medicine 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, as amended, including, but not limited to, express or implied statements regarding Disc's expectations, hopes, beliefs, intentions or strategies with respect to its AURORA Phase 2 and BEACON Phase 2 clinical studies of bitopertin and the results thereof, its Phase 1b/2 clinical studies of DISC-0974 in patients with MF and NDD-CKD patients with anemia, its Phase 1 clinical study of DISC-3405 in healthy volunteers, projected timelines for the initiation and completion of its clinical trials, anticipated timing of release of data and other clinical activities, Disc's business plans and objectives, Disc's analysis of market potential for patients with EPP, and Disc's beliefs about operating expenses and that it will have capital to fund Disc well into 2026. The use of words such as, but not limited to, "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "future," "intends," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "seek," "suggest," "will," or "would" 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 other risks and uncertainties described in the "Risk Factors" section of our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, and other documents filed by Disc from time to time with the Securities and Exchange Commission (SEC), as well as discussions of potential risks, uncertainties, and other important factors in Disc's subsequent filings with the SEC. 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.

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