Disc Medicine Announces Successful Type C Meeting with FDA for Bitopertin in Erythropoietic Protoporphyria (EPP) and Shares Plans for NDA Submission

January 21, 2025

- *Pursuing accelerated approval for bitopertin in EPP with protoporphyrin IX (PPIX) reduction as the surrogate endpoint*
- *Planning to submit NDA under accelerated approval pathway in H2 2025 based on existing clinical data, including results from BEACON and AURORA Phase 2 trials*
- *Achieved regulatory alignment on APOLLO post-marketing confirmatory trial design and on track to initiate trial by mid-year 2025*
- *Aligned on average monthly time in light without pain during the last month of the 6-month treatment period and percent change from baseline in whole-blood metal-free PPIX after 6 months of treatment as coprimary endpoints for confirmatory trial*
- *Management will host a conference call on Tuesday, January 21 at 8:00 am EST.*

WATERTOWN, Mass., Jan. 21, 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 announced positive feedback from its Type C meeting with the U.S. Food and Drug Administration (FDA) to discuss the APOLLO post-marketing confirmatory trial for bitopertin in EPP.

“Our recent FDA interaction marks another step toward delivering a potentially life-altering therapy for EPP patients, and we appreciate the collaboration with regulators, our investigators, and the EPP patient community which has brought us to this point,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc. “Last year, we aligned with the FDA on PPIX reduction as a surrogate endpoint for potential accelerated approval of bitopertin, and we are actively pursuing that path with plans to submit an NDA in the second half of 2025. As part of that process, the Type C meeting has provided further clarity on our plans for the APOLLO post-marketing confirmatory trial, which will kick off by the middle of this year and could eventually be the basis for converting an accelerated approval, if granted, to a full approval.”

The meeting resulted in alignment on the design of the APOLLO post-marketing confirmatory trial. Key features include:

- Co-primary endpoints of average monthly total time in sunlight without pain between 10:00 and 18:00 during the last month of the 6-month treatment period and percent change from baseline in whole blood metal-free PPIX after 6 months of treatment;
- Other measures of efficacy such as occurrence of phototoxic reactions, cumulative total pain-free time in sunlight, patient global impression of change (PGIC), and time to prodrome;
- Selection of 60 mg dose of bitopertin and 6-month treatment duration;
- Inclusion of patients aged 12+ with EPP including X-linked protoporphyria (XLP); and
- Double-blind, placebo-controlled study with ~150 patients randomized 1:1.

Disc plans to initiate the APOLLO trial by mid-2025 and will include sites in the US, Canada, Europe, and Australia. Based on guidance toward an NDA submission in H2 2025, Disc expects enrollment of the APOLLO trial to be well underway by the time of an accelerated approval, if granted.

Management will host a call to discuss these updates on Tuesday, January 21 at 8:00 am EST. Please register for the event on the Events and Presentations page of Disc’s website (https://ir.discmedicine.com/).

About Bitopertin

Bitopertin is an investigational, clinical-stage, orally administered inhibitor of glycine transporter 1 (GlyT1) that is designed to modulate heme biosynthesis. GlyT1 is a membrane transporter expressed on developing red blood cells and is required to supply sufficient glycine for heme biosynthesis and support erythropoiesis. Disc is planning to develop bitopertin as a potential treatment for a range of hematologic diseases including erythropoietic porphyrias, where it has potential to be the first disease-modifying therapy. Bitopertin has been studied in multiple clinical trials in patients with EPP, including the Phase 2 open-label BEACON trial, the Phase 2 double-blind, placebo-controlled AURORA trial, and an open-label extension HELIOS trial.

Bitopertin is an investigational agent and is not approved for use as a therapy in any jurisdiction worldwide. Disc obtained global rights to bitopertin under a license agreement from Roche in May 2021.

About Erythropoietic Protoporphyria (EPP)

Erythropoietic protoporphyria (EPP), including X-linked Protoporphyria (XLP), is a rare, debilitating and potentially life-threatening disease caused by mutations that affect heme biosynthesis, resulting in the accumulation of a toxic, photoactive intermediate called protoporphyrin IX (PPIX). This causes severe reactions when patients are exposed to sunlight, characterized by excruciating pain, edema, burning sensations and potential blistering and disfigurement. PPIX also accumulates in the hepatobiliary system and can result in complications including gallstones, cholestasis, and liver damage in 20-30% of patients and in extreme cases liver failure. Current standard of care involves extreme measures to avoid sunlight, including restricting outdoor activities to nighttime, use of protective clothing and opaque shields, and pain management. This has a significant impact on the psychosocial development, quality of life, and daily activities of patients, particularly in young children and families. There is currently no cure for EPP and only one FDA-approved therapy, a surgically implanted synthetic hormone designed to stimulate melanin production called Scenesse® (afamelanotide).

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 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 its potential APOLLO confirmatory clinical study of bitopertin in EPP and XLP patients, including the proposed study design, the anticipated timeline, and the results thereof; and the possible regulatory path forward for bitopertin in EPP, including whether the FDA will determine that the accelerated approval pathway continues to be appropriate, the treatment of the APOLLO clinical study as a post-marketing confirmatory trial, and the timeline for a potential NDA submission and accelerated approval, if granted, and whether the NDA submission will meet the standards of accelerated approval. 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 its Annual Report on Form 10-K for the year ended December 31, 2023, and in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2024. 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