Disc Medicine Announces Successful End of Phase 2 Meeting with FDA for Bitopertin in Erythropoietic Protoporphyria (EPP), Including Potential for Accelerated Approval

November 4, 2024

- *Alignment with the FDA across all proposed study parameters, providing a clear development path to registration*
- *Agreement on proposed primary endpoint of average monthly time in sunlight during the last month following a 6-month treatment period*
- *Potential for accelerated approval based on existing data and utilizing reduction of PPIX as a surrogate endpoint*
- *Plan to initiate APOLLO trial, a 6-month study of a 60 mg dose of bitopertin in EPP and XLP patients ages 12+ by mid-2025*
- *Management will host a conference call on Monday, November 4 at 8:00 am EST*

WATERTOWN, Mass., Nov. 04, 2024 (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 end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA), supporting the regulatory path forward for bitopertin in EPP.

“We are thrilled with the outcome of our end-of-Phase 2 meeting with the FDA, which provides us with a clear development path forward for bitopertin. Importantly, the FDA agreed with all attributes of our study design, including a primary endpoint we feel is statistically robust and would fully capture the potential benefit of bitopertin in EPP,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc. “We’re particularly excited by the potential to file under the Accelerated Approval Program based on our existing data and use of PPIX reduction as a surrogate endpoint. This is a testament to the significant unmet need in EPP and the strength of the bitopertin data package, and we look forward to engaging further with the FDA on this pathway.”

The meeting resulted in agreement on all proposed attributes of the APOLLO study, which the company plans to initiate by mid-2025, including the:

- Sufficiency of a single, randomized, double-blind, placebo-controlled trial;
- Primary endpoint of average monthly total time in sunlight without pain during the last month following 6 months of treatment;
- Additional measures such as change in PPIX, occurrence of phototoxic reactions, cumulative total pain-free time in sunlight, and patient global impression of change (PGIC);
- Selection of 60 mg dose of bitopertin and 6-month treatment duration; and
- Inclusion of patients aged 12+ with EPP, including X-linked protoporphyria (XLP).

In addition, the FDA also agreed with the potential for reduction of PPIX to serve as a surrogate endpoint to support an accelerated approval. Under this pathway, Disc would have the potential to submit an NDA based on the existing data package and the APOLLO trial would serve as a confirmatory trial. Disc will be meeting with the FDA to finalize the details of APOLLO and plans to provide an update in Q1 2025 on this discussion as well as timing for NDA filing under an accelerated pathway.

Management will host a call to discuss these updates on Monday, November 4 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) and X-linked Protoporphyria (XLP)

Erythropoietic protoporphyria (EPP) and X-linked Protoporphyria (XLP) are rare, debilitating and potentially life-threatening diseases 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 clinical study of bitopertin in EPP and XLP patients, including the proposed study parameters, the anticipated timeline, and the results thereof; and the possible regulatory path forward for bitopertin in EPP, including the potential to seek approval under the Accelerated Approval pathway and conduct a confirmatory trial, and the timeline of related discussions with the FDA. 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.

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