Disc Medicine Announces Submission of New Drug Application (NDA) to US FDA for Accelerated Approval of Bitopertin for Patients with Erythropoietic Protoporphyria (EPP)

September 30, 2025

- *Disc is seeking accelerated approval and priority review of its NDA submission*
- *FDA decision to accept and file the NDA for review occurs within 60 days of submission*

WATERTOWN, Mass., Sept. 30, 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 the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for bitopertin for patients aged 12 years and older with erythropoietic protoporphyria (EPP), including X-linked protoporphyria (XLP). Disc submitted the NDA under the FDA's accelerated approval pathway using reduction of protoporphyrin IX (PPIX) as a surrogate endpoint and requested a Priority Review based on bitopertin's potential to address the significant unmet need for EPP patients. Bitopertin has received Orphan Drug Designation and Rare Pediatric Disease Designation from the FDA.

"This NDA submission represents a pivotal moment not just for Disc but for the EPP community as we seek to provide patients with a treatment option that has the potential to address the underlying cause of disease," said John Quisel, J.D., Ph.D., Chief Executive Officer and President of Disc. "We look forward to working closely with regulators throughout the review process and remain focused on preparations for bitopertin's anticipated launch. I want to express our gratitude to our dedicated investigators and the patients who participated in our clinical trials, and their families and caregivers who helped make this all possible."

The NDA submission is supported by the results of the Phase 2 BEACON and AURORA studies in EPP, as well as prior data generated by Roche, including a safety database of over 4,000 clinical trial participants. The BEACON and AURORA studies demonstrated significant reductions in PPIX and improvements across key aspects of the disease, including improvements in light tolerance, reduction of phototoxic reactions, and improvements in quality of life. Disc is also studying bitopertin in a long-term extension study, HELIOS, and initiated the APOLLO confirmatory study in April 2025.

The NDA includes a request for Priority Review, which, if granted, would accelerate the timing of the FDA's goal for review of the application to six months following the end of the 60-day filing review period rather than the standard 10-month review period. Priority Review status is designated for drugs that may offer a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition.

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, an open-label extension HELIOS trial, and the confirmatory Phase 3 double-blind, placebo-controlled APOLLO 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 (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 registrational pathway for bitopertin, including the timeline for the FDA's review of the NDA submission, the timing of the FDA's filing decision, the potential for a priority review, and the potential for accelerated approval; the potential of bitopertin as a therapeutic drug; and the potential commercial launch of bitopertin. 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 SEC, 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,

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