Disc Medicine Receives Complete Response Letter from FDA for Bitopertin for the Treatment of EPP

February 13, 2026

- FDA acknowledged that AURORA and BEACON provided sufficient evidence that bitopertin significantly lowers PPIX and that there is a strong mechanistic and biological plausibility supporting the use of the PPIX biomarker in protoporphyria
- FDA indicated a need to see the results of the ongoing Phase 3 APOLLO study before making a decision
- Ongoing Phase 3 APOLLO study potential to serve as basis for traditional approval; topline data anticipated Q4 2026

WATERTOWN, Mass., Feb. 13, 2026 (GLOBE NEWSWIRE) -- Disc Medicine, Inc. (NASDAQ:IRON), a biopharmaceutical company focused on the discovery, development, and commercialization of novel treatments for patients suffering from serious hematologic diseases, announced that the U.S. Food and Drug Administration (FDA) today issued a Complete Response Letter (CRL) for the New Drug Application (NDA) for bitopertin as a treatment for patients with erythropoietic protoporphyria (EPP). Bitopertin has been under review for accelerated approval and as part of the Commissioner's National Priority Voucher (CNPV) pilot program.

Accelerated approval relies on (1) whether there is evidence of an effect on the proposed surrogate endpoint (% change in whole blood metal-free PPIX) and (2) whether the proposed surrogate endpoint, including the magnitude of change, is reasonably likely to predict a clinical benefit. On the first point, the FDA agreed that AURORA and BEACON provided sufficient evidence that bitopertin significantly lowers whole blood metal-free PPIX. On the second, based on review of AURORA and BEACON results, the FDA concluded that the trials did not show evidence of association between percent change in PPIX and sunlight exposure-based endpoints, as measured in the trials, despite the strong mechanistic and biological plausibility supporting the use of the PPIX biomarker in protoporphyria. The FDA indicated results of the APOLLO study could serve as evidence to support traditional approval.

"We are committed to delivering bitopertin to patients, knowing how critical this potentially disease-modifying therapy is to the EPP community. While our efforts at utilizing expedited pathways to get bitopertin to patients quickly have not come to fruition, we are continuing to pursue all avenues in support of FDA approval," said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc Medicine. "The CRL will delay the potential approval of bitopertin, but we have confidence in the ongoing APOLLO trial, for which we are seeing incredible enthusiasm from the EPP community. Confidence in our product and program guides our approach, and we will continue working closely with the FDA to support their review."

Disc believes the issue raised is readily addressable, given the APOLLO study is already well underway with topline data expected in Q4. Disc plans to request a Type A meeting to review our approach with the FDA. A blinded sample size re-estimation of the APOLLO study was conducted in January and no modifications to sample size were needed based on statistical analysis. There has been significant patient and physician enthusiasm around the APOLLO trial, allowing Disc to complete trial enrollment in March 2026, several months earlier than expected. Upon completion of APOLLO, Disc would then file a response to the CRL and expect an updated FDA decision by mid-2027. Disc has approximately $791 million at December 31, 2025 in unaudited cash, cash equivalents, and marketable securities and maintains guidance of providing runway into 2029.

Disc Medicine will host a call for investors at 8 am ET on Tuesday, February 17th to discuss this outcome. Please register for the event on the Events and Presentations page of Disc's website (https://ir.discmedicine.com/).

A copy of the CRL will be included in a Form 8-K to be filed with the Securities and Exchange Commission, which will be accessible on 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 developing 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 Disc Medicine

Disc Medicine (NASDAQ:IRON) is a 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: the APOLLO clinical trial, including timing for completion and the results thereof; the future regulatory path for bitopertin with the FDA, including the potential for traditional approval, the timing of any such approval, and the potential for APOLLO to serve as the basis for any such approval; and our 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 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, whether as result of new information, future events or otherwise, except as required by law.

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