Disc Presents Positive Initial Data from Phase 2 BEACON Trial of Bitopertin in Patients with Erythropoietic Protoporphyria (EPP) at European Hematology Association (EHA) 2023 Congress

June 9, 2023

- *Consistent and dose-dependent reductions of protoporphyrin IX (PPIX), the disease-causing metabolite in EPP, were observed in patients treated with bitopertin*
- *Patients reported significant improvements in sunlight tolerance and measures of quality-of-life*
- *Bitopertin was well-tolerated, with no meaningful changes in hemoglobin observed*
- *Disc Medicine to host an investor conference call today at 7:30 AM ET*

WATERTOWN, Mass., June 09, 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 preliminary findings from its ongoing, Phase 2 open-label BEACON trial evaluating bitopertin, an orally administered glycine transporter 1 (GlyT1) inhibitor, in patients with erythropoietic protoporphyria (EPP) and X-linked protoporphyria (XLP) at the European Hematology Association (EHA) 2023 Congress in Frankfurt, Germany. The initial trial data demonstrated consistent decreases in PPIX, significant increases in reported sunlight tolerance and improvements in measures of patient quality of life.

“We’re delighted to share these initial, positive data from BEACON, which provide the first clinical evidence supporting our therapeutic hypothesis of bitopertin in EPP. Over the next 12 months, we plan to build on this momentum with a series of additional clinical read-outs across our portfolio,” said John Quisel, J.D., Ph.D., Chief Executive Officer and President of Disc Medicine. “This is an important moment for Disc as a company, and I want to extend my gratitude to our team, collaborators, and most importantly, the patients and families participating in BEACON.”

“We are excited to share these initial data from the BEACON trial, where we observed consistent and sustained suppression of PPIX, the disease-causing metabolite in EPP, in patients treated with bitopertin,” said Will Savage, M.D., Ph.D., Chief Medical Officer at Disc Medicine. “Importantly, this reduction translated into significant improvements in the time that patients can spend in sunlight without reporting pain or symptoms related to their disease. We’re encouraged by the data and plan to present additional data at the end of the year.”

The BEACON trial is a randomized, open-label, parallel-arm trial enrolling up to 22 patients with EPP or XLP at trial sites in Australia. 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 trial is ongoing and these data reflect initial data from 15 subjects enrolled as of the data cutoff of May 8, 2023, with a range of treatment durations from 18 days to 6 months. Due to batch processing of samples, the data cutoff for PPIX data was April 7, 2023.

Highlights of the initial data presented:

- Protoporphyrin IX (PPIX) levels: Significant, consistent, dose-dependent, and sustained reductions of whole-blood, metal-free PPIX; mean reduction of >40% when compared to baseline
- Measures of light tolerance (individual) from two participants with the longest follow-up demonstrated substantial increases in sunlight tolerance as measured by time in sunlight without experiencing a prodrome (initial symptoms that signal a pain attack), or “sunlight challenge”:
    - A participant on 20 mg bitopertin reported a >80-fold increase in sunlight tolerance on day 88 of treatment, increasing from 4.5 minutes at baseline to over 6 hours; the participant did not report a prodrome during any sunlight challenge after Day 20
    - A participant on 60 mg bitopertin reported a >200-fold increase in sunlight tolerance on day 74 of treatment, increasing from 1.25 minutes at baseline to over 4 hours, and did not report a prodrome during any sunlight challenge after Day 120
- Measures of light tolerance (aggregated across participants from whom data was available in the trial):
    - Average weekly total time spent in sunlight: increased from 344 minutes (approximately 49 minutes per day) to 1,200 minutes at Week 24
    - Time to prodrome during sunlight challenge (averaged over a two-week period): increased >7-fold, from 25 minutes at baseline to 182 minutes at Week 24
    - Increased proportion of days without symptoms: 75% vs. 25% (baseline)

- Increased proportion of sunlight challenges without prodromes: 50% vs. 0% (baseline)
- Phototoxic reactions: 96% reduction in patient-reported phototoxic reactions while on treatment compared to baseline (n=15)

- Measures of patient quality of life
  - Patient Global Impression of Change (PGIC): All 10 patients that had completed a day 43 visit reported their disease was much better (n=8) or a little better (n=2) in the last 7 days
  - Patient Global Impression of Severity (PGIS): Nine out of 10 patients that had completed a day 43 visit reported their EPP was mild (n=3) or not at all severe (n=6)
  - EPP Impact Questionnaire (EPIQ): For patients whose most recent data was Day 43, 4/8 patients reported an improvement in the impact of EPP on quality of life and 4/8 reported no change in the impact of EPP on quality of life. For patients whose most recent data was after Day 43, 2/2 reported marked improvement in the impact of EPP on quality of life, reporting no impact of EPP on quality of life.
- Bitopertin was well-tolerated at both dose levels with no reported serious adverse events, no reported discontinuations or dose reductions, no reported adverse events greater than Grade 1, and no meaningful changes observed in mean hemoglobin levels

These data were presented at the European Hematology Association 2023 Congress in Frankfurt, Germany and the poster is available on the EHA Congress platform at www.ehaweb.org.

Management will host a call to review the presented data on Friday, June 9th at 7:30 am ET. 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. There are currently two ongoing Phase 2 clinical trials of bitopertin in patients with erythropoietic porphyria, including an open-label trial called BEACON and a randomized, double-blind placebo-controlled trial called AURORA.

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 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, including, but not limited to, express or implied statements regarding Disc's expectations with respect to its BEACON Phase 2 clinical trial of bitopertin and projected timelines for the initiation and completion of its clinical trials, the timing of additional data and other activities. The use of words such as, but not limited to, "aim," "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 data 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 data 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 data of Disc's preclinical studies and clinical trials and the risk that the data of Disc's preclinical studies and clinical trials may not be predictive of future data 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 filed with the Securities and Exchange Commission (SEC) on May 15, 2023 and other documents filed by Disc from time to time with the 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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