Disc Medicine Announces First Patient Enrolled in Phase 1/2 Clinical Trial of Bitopertin in Diamond-Blackfan Anemia (DBA)

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WATERTOWN, Mass., July 27, 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, announced today that the first patient has been enrolled in the National Institutes of Health-sponsored Phase 1/2 clinical trial of bitopertin in Diamond-Blackfan anemia (DBA). Bitopertin is an investigational, orally administered glycine transporter 1 (GlyT1) inhibitor designed to modulate heme biosynthesis.

“The start of this trial represents another step toward demonstrating the potential of bitopertin to address a wide range of hematologic conditions.” said Will Savage, M.D., Ph.D., Chief Medical Officer of Disc. “We are excited to test the potential of treating DBA by modulating heme synthesis with bitopertin, particularly in light of our recent positive initial data from an open label trial of bitopertin in erythropoietic protoporphyria (EPP) at EHA. We appreciate the support and leadership of the National Heart, Lung, and Blood Institute and look forward to continued collaboration on this trial.”

The Phase 1/2 study will be a single-arm, dose-escalation trial of bitopertin in DBA patients who either have steroid-refractory and/or relapsed disease or are unable to tolerate systemic corticosteroids. The study includes planned dose escalation within each participant to continually assess for hematologic response. Upon completion of the main treatment period, patients may continue on extended treatment within the trial. This study will be conducted and funded by the NIH under a Cooperative Research and Development Agreement (CRADA), under the direction of Dr. Cynthia Dunbar, M.D., the NIH Distinguished Investigator and Chief Translational Stem Cell Biology Branch, and Head, Molecular Hematopoiesis Section, NHLBI, with Dr. David Young, M.D., Ph.D., NHLBI Staff Clinician as Principal Investigator.

The content of this research is solely the responsibility of the authors and does not necessarily represent the official views of the National Institutes of Health.

About Diamond-Blackfan Anemia

Diamond-Blackfan Anemia (DBA) is a rare, inherited blood disorder characterized by the failure of bone marrow to produce red blood cells. The incidence of DBA is approximately 1:100,000 to 1:200,000 live births every year. Patients are usually diagnosed during infancy and commonly present with severe anemia, pallor, fatigue, as well as other potential abnormalities. DBA is chronic and requires lifelong management with corticosteroids and blood transfusions, both of which are associated with serious toxicities and long-term complications. Evidence suggests that the anemia of DBA may be caused by the accumulation of excess heme in developing red blood cells, which is toxic and leads to their premature death. Preclinical studies have shown that targeting elevated heme levels has the potential as a therapeutic strategy for DBA.

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 Disc

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, as amended, including, without limitation, express or implied statements related to Disc’s expectations regarding the timing and closing of the offering, and the anticipated use of proceeds from the offering. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “seek,” “target” and

similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release. These risks and uncertainties include fluctuations in Disc's stock price, changes in market conditions, the satisfaction of customary closing conditions related to the public offering, and other risks identified in our SEC filings, including our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, and in the preliminary prospectus supplement related to the offering filed with the SEC on June 12, 2023. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

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