Disc Medicine Announces Collaboration with National Institutes of Health for Phase 2 Clinical Study of Bitopertin in Patients with Diamond-Blackfan Anemia (DBA)

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IND accepted for phase 2 clinical trial in DBA patients; study is expected to initiate mid-year 2023

WATERTOWN, Mass., March 21, 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 a collaboration with the National Heart Lung and Blood Institute (NHLBI) of the National Institutes of Health (NIH) to evaluate bitopertin, a therapeutic candidate designed to modulate heme biosynthesis, in a phase 2 clinical study of patients with Diamond-Blackfan anemia (DBA). The study will be conducted and funded by the NIH under a Cooperative Research and Development Agreement (CRADA) and is expected to initiate mid-year 2023.

“We’re thrilled to have the support of the NHLBI for this clinical trial as there is growing evidence to suggest that reducing excess levels of heme may be an effective treatment strategy for DBA. This collaboration will enable us to benefit from the experience of the NHLBI in designing and conducting clinical studies of DBA.” said John Quisel, J.D., Ph.D., Chief Executive Officer and President of Disc. “Disc now has ongoing development programs of bitopertin in both erythropoietic porphyria and DBA. We believe controlling heme synthesis has the potential to address a wide range of hematologic conditions and are planning studies in additional indications.”

Under the CRADA, the NHLBI will serve as the regulatory sponsor and be responsible for conducting a phase 2 clinical study of bitopertin in DBA patients. The study will be jointly funded by the NHLBI and Disc. The study will be 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.

The phase 2 study will be a pilot, 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.

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, 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 a clinical-stage, orally administered inhibitor of 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, Diamond-Blackfan Anemia (DBA) and others. There are currently two ongoing phase 2 clinical studies 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 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, 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 collaboration with the NIH and the Phase 2 clinical study of Bitopertin in patients with DBA and projected timelines for the initiation and completion of its clinical trials and other activities. 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 other risks and uncertainties described in the "Risk Factors" section of the Current Report on Form 8-K filed with the SEC on December 29, 2022 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 Securities and Exchange Commission. 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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