Disc Medicine Initiates a Phase 1b/2 Clinical Study of DISC-0974 in Adults with Non-Dialysis Dependent Chronic Kidney Disease (NDD-CKD) and Anemia

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WATERTOWN, Mass., Feb. 16, 2023 (GLOBE NEWSWIRE) -- Disc Medicine, a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel treatments for patients suffering from serious hematologic diseases, announced today the initiation of a Phase 1b/2 clinical study of DISC-0974 in non-dialysis dependent chronic kidney disease (NDD-CKD) patients with anemia. DISC-0974 is a monoclonal antibody designed to suppress hepcidin by inhibiting the hemojuvelin (HJV) co-receptor, and thereby address anemia by enhancing the availability of iron for erythropoiesis. DISC-0974 is also currently being studied in a Phase 1b/2 clinical study in patients with myelofibrosis and anemia.

“We are excited to initiate this clinical trial of DISC-0974 in chronic kidney disease, where hepcidin plays a key role in the pathophysiology of anemia. There is a tremendous need for innovative therapies that work through mechanisms outside of the erythropoietin pathway,” said John Quisel, JD, PhD, Chief Executive Officer at Disc Medicine. “We believe DISC-0974 has potential across a broad range of chronic and inflammatory diseases. With the start of this study, we now have ongoing clinical trials for DISC-0974 in both chronic kidney disease and myelofibrosis, and plan to explore its use in other indications as well.”

The study will be a multi-center, Phase 1b/2 trial and will evaluate the safety, tolerability, and efficacy of DISC-0974 in NDD-CKD patients with anemia. The study endpoints will include hepcidin levels, serum iron and markers of iron mobilization and changes in hemoglobin. The study enrollment criteria will include patients with Stage II-V CKD not receiving dialysis, baseline Hb < 11.0 g/dL for males and Hb < 10.5 g/dL for females, and those not receiving concurrent treatment with erythropoietin-stimulating agents (ESAs). The study will be conducted in two parts:

- *Phase 1b (Dose-Escalation):* Randomized, double-blind, placebo-controlled study design; single, ascending doses of DISC-0974 will be administered subcutaneously at the following planned dose levels: 28 mg, 40 mg, 60 mg, 90 mg; safety, PK and hematologic effects will be assessed at each dose level; a dose for the expansion phase will be selected based on optimal increases in hemoglobin;
- *Phase 2 (Expansion Stage)*: Open-label, single-arm study design; multiple doses of DISC-0974 administered subcutaneously, once-monthly at the dose level selected from the phase 1b portion of the study for three months.

“There are few options available for the treatment of CKD anemia, particularly in the non-dialysis setting where it affects millions of patients and the majority do not receive any treatment for their anemia,” said Steven Fishbane, MD, Professor of Medicine at Donald and Barbara Zucker School of Medicine at Hofstra/Northwell. “Elevated hepcidin has long been recognized as an important driver of CKD anemia. I’m excited by the initiation of this study of DISC-0974 in NDD-CKD patients and look forward to the results.”

About DISC-0974

DISC-0974 is an investigational monoclonal antibody (mAb) targeting a BMP-signaling co-receptor called hemojuvelin (HJV) and is designed to suppress hepcidin production and increase serum iron levels in patients suffering from anemia of inflammation. DISC-0974 was in-licensed by Disc from AbbVie in 2019. Anemia of inflammation arises from abnormally elevated hepcidin and is the most common form of anemia, affecting millions of patients in the US across numerous diseases such as chronic kidney disease, myelofibrosis, cancer, autoimmune diseases, and other conditions with an inflammatory component. Disc has established clinical proof-of-mechanism of DISC-0974 in a Phase 1 trial of healthy volunteers and initiated a Phase 1b/2a clinical trial of DISC-0974 in patients with myelofibrosis and anemia, as well as a Phase 1b/2a clinical trial of DISC-0974 in patients with chronic kidney disease and anemia who are not receiving dialysis.

DISC-0974 is an investigational agent and is not approved for use as a therapy in any jurisdiction worldwide.

About Anemia of Chronic Kidney Disease (CKD)

Chronic kidney disease (CKD) is a global, widespread disease characterized by progressive loss of kidney function and may lead to end-stage renal disease (ESRD) or kidney failure. CKD affects over 37 million patients in the United States and an estimated 700 million patients worldwide. Anemia is a serious and frequent complication of CKD, as patients are unable to produce sufficient red blood cells and hemoglobin. It affects approximately 5-6 million patients in the U.S. alone, may results in fatigue, shortness of breath, and reduced physical and cognitive function, and can be associated with a higher risk of mortality, hospitalization and other complications. Elevated hepcidin is a primary cause of anemia in CKD patients and prevents erythropoiesis by depriving developing red blood cells of iron. Hepcidin accumulates at high levels in CKD patients because its production is stimulated by inflammation and its clearance is reduced as a consequence of impaired renal function. The majority of CKD patients do not receive any treatment for their anemia due to the complexity of outpatient administration and potential safety concerns related to the current treatments. In severe cases, patients may receive blood transfusions, but use may lead to increased administrative burden, safety risks and the potential for immune sensitization which precludes eligibility for kidney transplantation.

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, 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 Phase 1b/2 clinical study of DISC-0974 in NDD-CKD patients with anemia 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.

Media Contact

Peg Rusconi
Verge Scientific Communications
prusconi@vergescientific.com

Investor Relations Contact

Suzanne Messere
Stern Investor Relations
suzanne.messere@sternir.com