Disc Medicine Receives FDA Orphan Drug Designation for DISC-3405 for the Treatment of Polycythemia Vera

February 9, 2024

WATERTOWN, Mass., Feb. 09, 2024 (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 announced that the United States Food and Drug Administration (FDA) has granted Orphan Drug Designation to DISC-3405 for the treatment of patients with polycythemia vera (PV).

“Orphan drug designation is an important milestone that highlights the potential of DISC-3405 in PV, a rare disease with few treatment options,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc. “We look forward to sharing initial data from our ongoing Phase 1 trial of DISC-3405 in healthy volunteers in the first half of 2024.”

FDA Orphan Drug Designation is granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States. Orphan Drug status provides benefits to drug developers, including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of post-approval marketing exclusivity.

About DISC-3405

DISC-3405 is an investigational, anti-TMPRSS6 (Transmembrane Serine Protease 6, also known as Matriptase-2) monoclonal antibody designed to increase hepcidin production and suppress serum iron. Disc in-licensed DISC-3405 from Mabwell Therapeutics in January 2023. Preclinical studies of DISC-3405 have demonstrated an increase in hepcidin production and suppression of serum iron levels in animal models of beta-thalassemia and polycythemia vera. Disc initiated a Phase 1 study of DISC-3405 in healthy volunteers in October 2023 and plans to develop DISC-3405 initially as a treatment for polycythemia vera as well as other hematologic conditions.

DISC-3405 is an investigational agent and is not approved for use as a therapy in any jurisdiction worldwide.

About Polycythemia Vera (PV)

Polycythemia vera (PV) is a chronic and rare myeloproliferative neoplasm characterized by the abnormal proliferation of red blood cells. PV affects approximately 150,000 patients in the U.S. and has a similar prevalence in Europe. The overproduction of red blood cells alters the viscosity of blood, causing it to thicken and placing patients at an elevated risk of cardiovascular and thromboembolic events, such as heart attack and stroke. Patients also experience complications such as enlarged spleen and symptoms of their disease such as fatigue, pruritis, difficulty concentrating and others. Current therapy involves phlebotomy to physically remove blood and iron to limit erythropoiesis or treatment with cytoreductive agents, with the goal of reducing red blood cell count and managing symptoms.

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, 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 Disc’s expectations with respect to DISC-3405 and its clinical development. 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: Disc’s expectations regarding the clinical development and commercialization of

DISC-3405; Disc's expectations regarding future growth and innovation; the other risks and uncertainties described in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2022, Quarterly Reports on Form 10-Q for the quarters ended March 31, 2023, June 30, 2023, and September 30, 2023 and other documents filed by Disc from time to time with the Securities and Exchange Commission (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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