Disc Medicine Presents Positive Updated Results from Phase 1b Trial in Patients with Myelofibrosis (MF) and Anemia in an Oral Presentation at the 66th American Society of Hematology (ASH) Annual Meeting

December 9, 2024

- *Demonstrated durable hematologic response across all patient subgroups, regardless of baseline transfusion status and concomitant JAK inhibitor therapy*
- *Phase 2 study in MF anemia has been initiated, enrolling a broad range of patient types*

WATERTOWN, Mass., Dec. 08, 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 presented positive updated results from a Phase 1b trial of DISC-0974 in patients with myelofibrosis (MF) and anemia. The data, presented in an oral session at the 2024 American Society of Hematology (ASH) annual meeting in San Diego, CA, demonstrated that treatment with DISC-0974 results in substantial reductions in hepcidin and increases in iron levels translating to positive impact on clinically meaningful measures of anemia across a broad range of patient types.

“With the presentation of this expanded data set from our phase 1b study, we are encouraged by the continued demonstration of robust hematologic activity of DISC-0974, including durable hemoglobin increases in all patient subgroups and meaningful reductions in transfusion burden. Importantly, we observed strong responses regardless of patients’ baseline transfusion burden or concomitant use of JAK inhibitors,” said John Quisel, JD, PhD, President and Chief Executive Officer of Disc Medicine. “With this data in hand, I’m pleased to announce that we have now started a phase 2 trial in myelofibrosis patients with anemia.”

This Phase 1b multi-center, open-label study, enrolled 35 adult patients with MF and anemia, including patients who were: non-transfusion dependent receiving no transfusions (nTD, n=23), transfusion dependent with low transfusion burden (TD Low, n=5) and transfusion dependent with high transfusion burden (TD High, n=7). The trial was comprised of both patients receiving concomitant JAK inhibitor therapy (n=13) and not receiving JAK inhibitor therapy (n=22). DISC-0974 was administered subcutaneously at 14 mg (n=1), 28 mg (n=7), 50 mg (n=12), 75 mg (n=9), or 100 mg (n=6) every 4 weeks for up to 6 treatments. Results demonstrated:

- Consistent, substantial decreases in hepcidin reaching >75% reduction from baseline and corresponding increases in serum iron across patients, which translated to increased levels of reticulocyte hemoglobin and hemoglobin
- 68% of baseline nTD patients achieved a hemoglobin increase of ≥1.5 g/dL during study period and 50% had sustained increases for ≥12 weeks
- 100% of TD patients with 1-2 transfusions within a 12-week period at baseline (TD Low) achieved a ≥50% reduction in transfusion requirement
    - 80% of TD Low patients achieved transfusion independence (TI) over a 16-week period
- 60% of TD patients with 3-12 transfusions within a 12-week period at baseline (TD High) achieved a ≥50% reduction in transfusion requirement
    - 40% of TD High patients achieved transfusion independence over a 12-week period
- 54% of patients receiving concomitant JAK inhibitor therapy achieved a major hematologic response
- DISC-0974 was generally well-tolerated at all evaluated dose levels. Diarrhea was the only adverse event (AE) that was considered related to DISC-0974 and reported in two or more subjects. The majority of AEs were not considered related to DISC-0974.

Management will host a call today, Sunday, December 8 at 9:00pm EST / 6:00pm PST to review highlights of data presented throughout the meeting and plans for next steps in development. Please register for the event on the Events and Presentations page of Disc’s website (https://ir.discmedicine.com/).

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 second 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, completed a Phase 1b clinical trial in patients with myelofibrosis and anemia, and initiated a Phase 2 clinical trial of DISC-0974 in patients with MF 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 Myelofibrosis

Myelofibrosis (MF) is a rare, chronic blood cancer that currently affects an estimated 25,000 patients in the United States alone. Severe, progressive, and treatment resistant anemia is the primary clinical manifestation of MF. At diagnosis, over 80% of MF patients have anemia, which progressively worsens and ultimately renders the majority of patients dependent on chronic red blood cell transfusions. Recent studies have shown hepcidin to be a key molecular driver of anemia in myelofibrosis. Hepcidin is elevated by approximately 12-fold in MF patients, and is correlated with disease severity, anemia, and the need for red blood cell transfusions.

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 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 clinical study of DISC-0974 in patients with MF and anemia, including the results thereof. 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; and the other risks and uncertainties described in Disc's filings with the Securities and Exchange Commission, including in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2023, and in subsequent Quarterly Reports on Form 10-Q. 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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