Disc Medicine Presents Positive Clinical Data Across Portfolio at the European Hematology Association (EHA) 2024 Congress

June 14, 2024

- *Updated analyses of data from AURORA and BEACON studies further demonstrate clinical activity of bitopertin across multiple measures of erythropoietic protoporphyria (EPP) and support development path forward*
- *Additional data from the ongoing Phase 1b trial of DISC-0974 in myelofibrosis (MF) patients continued to demonstrate greater than 60% hematologic response rates, with durable increases in hemoglobin levels and reductions in transfusion burden*
- *Positive data from initial single-ascending dose (SAD) cohorts of a Phase 1 trial of DISC-3405 in healthy volunteers demonstrated sustained, meaningful induction of hepcidin and >50% suppression of mean serum iron*

WATERTOWN, Mass., June 14, 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 positive additional data for bitopertin in erythropoietic protoporphyria (EPP), including additional analyses of AURORA data that further demonstrated the clinical activity of bitopertin and highlighted meaningful improvements in light tolerance, phototoxic reactions, and quality of life. Additionally, Disc shared updated data from a Phase 1b trial of DISC-0974 in MF anemia which demonstrated durable increases in hemoglobin and reduced transfusion burden in a majority of evaluable patients. Disc also shared initial SAD data from a Phase 1 healthy volunteer trial of DISC-3405 that provided proof of mechanism, showing that the drug has the potential to provide deep and sustained increases in hepcidin and reductions in iron. The data were presented at the European Hematology Association (EHA) 2024 Congress.

“This has been a tremendously exciting EHA meeting for Disc, where we had the opportunity to present data from ongoing clinical studies across our entire portfolio, showcasing the activity and therapeutic potential of each of our programs,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc. “At this meeting, we shared a deeper analysis of data from AURORA that underscores our confidence in bitopertin’s potential in EPP and supports a development path forward. We also provided an update from our study of DISC-0974 in anemia of myelofibrosis, which continues to demonstrate promising hematologic activity similar to what we showed at ASH. And, finally, we’re excited to share for the first time, data from our third program, DISC-3405, in healthy volunteers, demonstrating clinical proof-of-mechanism as an iron restriction agent with the potential for a differentiated profile.”

Bitopertin in EPP

The AURORA study is a randomized, double-blind, placebo-controlled Phase 2 clinical trial that enrolled 75 adult subjects with EPP. Subjects were randomized 1:1:1 to receive 20 mg of bitopertin (n=26), 60 mg of bitopertin (n=25), or placebo (n=24) once daily for 17 weeks. Key AURORA data presented include:

- Significant reductions in protoporphyrin IX (PPIX) (40% for the 60 mg group) compared to placebo
- Meaningful improvements on key aspects of EPP
  - Time-dependent improvement in light tolerance that was nominally significant compared to placebo in both the 20 mg (p=0.026) and 60 mg (p=0.013) dose groups
  - ~2x improvement in light tolerance relative to baseline in both 20 mg and 60 mg dose groups as evaluated in a post-hoc longitudinal analysis
  - Substantial, dose-dependent reductions in rate of phototoxic reactions compared to placebo, reaching statistical significance in the 60 mg dose group (75.3% reduction, p=0.011)
  - Dose-dependent improvements in Patient Global Impression of Change (PGIC), which were statistically significant for the 60 mg dose (p=0.022)
- Evaluation of the time course of phototoxic reactions and sunlight exposure showed greater treatment effect in the time period after PPIX nadir was reached, including elimination of observed phototoxic reactions in the 60 mg dose group
- Greater PPIX reductions were associated with improvements in multiple light tolerance measures, including cumulative total time in light, average time in sunlight without pain, change from baseline in time to prodrome, as well as PGIC
- Bitopertin was generally well-tolerated with no reported serious adverse events (SAEs) to date
- Disc also presented the full adult data set from BEACON, which was consistent with previously-presented results and demonstrated similar clinical activity to that observed in AURORA

DISC-0974 in MF

The Phase 1b/2a multi-center, open-label, ascending-dose clinical trial of DISC-0974 is enrolling patients with MF and severe anemia, including both transfusion and non-transfusion dependent patients. In the phase 1b dose-escalation phase, DISC-0974 is administered subcutaneously every 4 weeks for up to 6 treatments. Updated data from 34 patients with an April 29, 2024 cutoff include:

- Substantial and sustained reductions in hepcidin levels and increases in iron were observed in patients for several weeks

after each dose

- Strong hematologic response was observed across all patient types at 28-100 mg doses:
  - 68.9% of non-transfusion dependent (nTD) participants demonstrated a hemoglobin response of ≥1.5 g/dL (n=29)
  - 60% of nTD participants who have completed at least 16 weeks of treatment had a mean hemoglobin response of ≥1.5 g/dL above baseline sustained for at least 12 weeks (n=15)
  - One of two evaluable transfusion dependent (TD) participants became transfusion independent (TI) by the end of the trial
  - Hemoglobin response of ≥1.5 g/dL above baseline was achieved in 6 of 10 participants with concomitant JAK inhibitor therapy
  - All evaluable participants with baseline transfusion requirements demonstrated at least a 50% reduction in transfusions over a rolling 8-week window on trial compared to baseline (n=8)
- DISC-0974 was generally well-tolerated at all evaluated dose levels

DISC-3405

Initial data were also presented from the SAD portion of the Phase 1 clinical trial of DISC-3405 in healthy volunteers. In this trial, healthy males and females ages 18 to 65 were given a single dose of placebo (n=10) or DISC-3405 at 75 mg intravenously (IV) (n=6), 37.5 mg subcutaneously (SC) (n=6), 75 mg SC (n=6), 150 mg SC (n=6), or 300 mg SC (n=6). This initial data showed:

- A meaningful dose-dependent increase in hepcidin and corresponding reduction in serum iron across all dose levels
- Mean serum iron reduction in excess of 50% from baseline was achieved in the150- and 300-mg dose groups
- Mean serum iron reduction in excess of 50% was sustained for at least 4 weeks for the 300-mg dose group, with meaningful reduction observed in selective hematological parameters (CHr, hemoglobin, and hematocrit)
- PK/PD profile is supportive of monthly subcutaneous dosing.
- DISC-3405 was generally well-tolerated with no SAEs, AEs higher than Grade 2, or AEs leading to trial withdrawal reported to date

Management will host a call to review the presented data on Friday, June 14th at 8:00 am ET. Please register for the event on the Events and Presentations page of Disc's website (https://ir.discmedicine.com/).

Bitopertin, DISC-0974, and DISC-3405 are investigational agents and are not approved for use as therapies in any jurisdiction worldwide.

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 Medicine 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 AURORA Phase 2 and BEACON Phase 2 clinical trials of bitopertin and the results thereof, its Phase 1b/2 clinical trial of DISC-0974 in patients with MF and NDD-CKD patients with anemia, its initial SAD data in its Phase 1 clinical trial of DISC-3405 in healthy volunteers; and projected timelines for the initiation and completion of its clinical trials, anticipated timing of release of data, and other clinical 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 our Quarterly Report for the quarter ended March 31, 2024, 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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