Geron Announces FDA Acceptance of New Drug Application for Imetelstat for the Treatment of Lower Risk MDS

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FOSTER CITY, Calif.--(BUSINESS WIRE)-- Geron Corporation (Nasdaq: GERN), a late-stage clinical biopharmaceutical company, today announced that the United States Food and Drug Administration (FDA) has accepted the filing of Geron's New Drug Application (NDA) for imetelstat, its first-in-class telomerase inhibitor, for the treatment of transfusion-dependent anemia in patients with lower risk myelodysplastic syndromes (MDS).

"The FDA's acceptance of our New Drug Application is an important landmark along our steadfast journey to bring telomerase inhibition with imetelstat to the market," said John A. Scarlett, M.D., Geron's Chairman and Chief Executive Officer. "We look forward to continuing our collaboration with the FDA toward the goal of bringing imetelstat to the many patients for whom we believe this treatment could make a significant difference."

"FDA acceptance of our NDA is a significant milestone for both Geron and the MDS community, as there remain few treatment options and significant unmet needs, particularly for patients with difficult-to-treat subtypes of this cancer," said Faye Feller, M.D., Executive Vice President, Geron's Chief Medical Officer. "We believe that the IMerge Phase 3 data reflect the truly unique attributes of imetelstat, and, if approved, we expect imetelstat will change the standard of care in lower risk MDS."

The NDA submission is based on results from IMerge Phase 3, in which the primary endpoint of 8-week transfusion independence (TI) was significantly higher with imetelstat vs. placebo ($p<0.001$), with median TI duration approaching one year for imetelstat 8-week TI responders. Mean hemoglobin levels in imetelstat-treated patients increased significantly ($p<0.001$) over time compared to placebo patients. Statistically significant and clinically meaningful efficacy results were achieved across key MDS subgroups irrespective of ring sideroblast (RS) status, baseline transfusion burden and IPSS risk category. Patient-reported outcomes (PRO) data reported a sustained meaningful improvement in fatigue for imetelstat-treated patients vs. placebo. Safety results were consistent with prior imetelstat clinical experience.

As allowed under the 21st Century Cures Act, the FDA has up to 74 days from the NDA submission date to notify Geron of the Prescription Drug User Fee Act (PDUFA) action date for the NDA. Upon receipt of this notification, Geron plans to disclose the timeline for the NDA review.

Additionally, Geron expects to submit a Marketing Authorization Application (MAA) in the European Union (EU) in the fourth quarter of 2023.

About IMerge Phase 3

The Phase 3 portion of the IMerge Phase 2/3 study is a double-blind, 2:1 randomized, placebo-controlled clinical trial to evaluate imetelstat in patients with IPSS Low or Intermediate-1 risk (lower risk) transfusion dependent MDS who were relapsed after, refractory to, or ineligible for, erythropoiesis stimulating agent (ESA) treatment, had not received prior treatment with either a HMA or lenalidomide and were non-del(5q). To be eligible for IMerge Phase 3, patients were required to be transfusion dependent, defined as requiring at least four units of packed red blood cells (RBCs), over an eight-week period during the 16 weeks prior to entry into the trial. The primary efficacy endpoint of IMerge Phase 3 is the rate of red blood cell transfusion independence (RBC-TI) lasting at least eight weeks, defined as the proportion of patients without any RBC transfusion for at least eight consecutive weeks since entry to the trial (8-week TI). Key secondary endpoints include the rate of RBC-TI lasting at least 24 weeks (24-week TI), the duration of TI and the rate of hematologic improvement erythroid (HI-E), which is defined under 2006 IWG criteria as a rise in hemoglobin of at least 1.5 g/dL above the pretreatment level for at least eight weeks or a reduction of at least four units of RBC transfusions over eight weeks compared with the prior RBC transfusion burden. A total of 178 patients were enrolled in IMerge Phase 3 across North America, Europe, Middle East and Asia.

About Imetelstat

Imetelstat is a novel, first-in-class telomerase inhibitor exclusively owned by Geron and being developed in hematologic malignancies. Data from non-clinical studies and clinical trials of imetelstat provide strong evidence that imetelstat targets telomerase to inhibit the uncontrolled proliferation of malignant stem and progenitor cells in myeloid hematologic malignancies resulting in malignant cell apoptosis and potential disease-modifying activity. Imetelstat has been granted Fast Track designation by the U.S. Food and Drug Administration for both the treatment of adult patients with transfusion dependent anemia due to Low or Intermediate-1 risk MDS that is not associated with del(5q) who are refractory or resistant to an erythropoiesis stimulating agent, and for adult patients with Intermediate-2 or High-risk MF whose disease has relapsed after or is refractory to janus associated kinase (JAK) inhibitor treatment. Imetelstat is currently not approved by any regulatory authority.

About Geron

Geron is a late-stage biopharmaceutical company pursuing therapies with the potential to extend and enrich the lives of patients living with hematologic malignancies. Our first-in-class telomerase inhibitor, imetelstat, harnesses

Nobel Prize-winning science in a treatment that may alter the underlying drivers of disease. Geron currently has two Phase 3 pivotal clinical trials underway evaluating imetelstat in lower risk myelodysplastic syndromes (LR MDS), and in relapsed/refractory myelofibrosis (MF). To learn more, visit **www.geron.com** or follow us on **LinkedIn**.

Use of Forward-Looking Statements

Except for the historical information contained herein, this press release contains forward-looking statements made pursuant to the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Investors are cautioned that such statements, include, without limitation, those regarding: (i) that Geron expects to submit a MAA in the EU in the fourth quarter of 2023; (ii) that imetelstat may alter the underlying drivers of disease and has the potential to demonstrate disease-modifying activity in patients; (iii) that imetelstat could make a significant difference to patients and has the potential to change the standard of care in lower risk MDS; (iv) that Geron plans to disclose the timeline for the NDA review upon notification from the FDA of the PDUFA date; and (v) other statements that are not historical facts, constitute forward-looking statements. These forward-looking statements involve risks and uncertainties that can cause actual results to differ materially from those in such forward-looking statements. These risks and uncertainties, include, without limitation, risks and uncertainties related to: (a) whether regulatory authorities permit the further development of imetelstat on a timely basis, or at all, without any clinical holds; (b) whether any future safety or efficacy results cause the benefit-risk profile of imetelstat to become unacceptable; (c) whether imetelstat actually demonstrates that it alters the underlying drivers of disease and has disease-modifying activity in patients; and (d) whether the FDA will approve imetelstat for the treatment of transfusion-dependent anemia in patients with lower risk MDS. Additional information on the above risks and uncertainties and additional risks, uncertainties and factors that could cause actual results to differ materially from those in the forward-looking statements are contained in Geron's filings and periodic reports filed with the Securities and Exchange Commission under the heading "Risk Factors" and elsewhere in such filings and reports, including Geron's quarterly report on Form 10-Q for the quarter ended June 30, 2023 and future filings and reports by Geron. Undue reliance should not be placed on forward-looking statements, which speak only as of the date they are made, and the facts and assumptions underlying the forward-looking statements may change. Except as required by law, Geron disclaims any obligation to update these forward-looking statements to reflect future information, events or circumstances.

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