Geron Announces EMA Validation of Marketing Authorization 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 European Medicines Agency (EMA) has validated the Marketing Authorization Application (MAA) for imetelstat, a first-in-class investigational telomerase inhibitor, for the treatment of transfusion-dependent anemia in patients with lower risk myelodysplastic syndromes (MDS). The MAA was submitted earlier this month and is now under regulatory review by the European Committee for Medicinal Products for Human Use (CHMP) under the centralized procedure, which applies to all 27 EU member states, Iceland, Norway and Liechtenstein. Review of the MAA is expected to be approximately 14 months.

“The EMA validation of the marketing authorization application for imetelstat brings us one step closer to potentially offering this first-in-class therapeutic to lower risk MDS patients in the EU suffering from anemia,” said John A. Scarlett, M.D., Geron’s Chairman and Chief Executive Officer. “Based on the clinical profile of imetelstat to date, we are optimistic about its potential to become a standard of care and address longstanding unmet needs of lower risk MDS patients.”

The MAA 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. Consistent with prior imetelstat clinical experience, the most common serious adverse events were primarily short-lived, manageable cytopenias.

The MAA validation follows the acceptance by the U.S. Food and Drug Administration (FDA) of a New Drug Application (NDA), which was assigned a Prescription Drug User Fee Act (PDUFA) action date of June 16, 2024.

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 investigational 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 myelofibrosis (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 clinical biopharmaceutical company pursuing therapies with the potential to extend and enrich the lives of patients living with hematologic malignancies. Our first-in-class investigational telomerase inhibitor, imetelstat, harnesses Nobel Prize-winning science in a treatment that may alter the underlying drivers of disease. The New Drug Application (NDA) for imetelstat in lower risk myelodysplastic syndromes (LR MDS), based on the results from the Phase 3 IMerge clinical trial, is currently under review by the United States Food and Drug Administration (FDA) with a Prescription Drug User Fee Act (PDUFA) target action date of June 16, 2024. In addition,

an MAA is under review in the European Union for the same proposed indication. Furthermore, Geron currently has an ongoing pivotal Phase 3 clinical trial evaluating imetelstat 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 review of the MAA in the EU to be approximately 14 months; (ii) that imetelstat may alter the underlying drivers of disease in myeloid hematologic malignancies and has the potential to demonstrate disease-modifying activity in patients; (iii) that imetelstat has the potential to become the standard of care and address longstanding unmet needs of lower risk MDS patients; and (iv) 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 and EMA 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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