



Geron Receives Positive Opinion from the EMA Committee for Orphan Medicinal Products for Orphan Drug Designation in the European Union for Imetelstat to Treat Myelodysplastic Syndromes

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- Designation would provide 10-year period of marketing exclusivity in the European Union after product approval
- Patients are currently enrolling in a Geron-sponsored Phase 3 clinical trial in lower risk myelodysplastic syndromes

MENLO PARK, Calif., July 01, 2020 (GLOBE NEWSWIRE) -- Geron Corporation (Nasdaq: GERN), a late-stage clinical biopharmaceutical company, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on the Company's application for orphan drug designation of its first-in-class telomerase inhibitor, imetelstat, as a potential treatment for myelodysplastic syndromes (MDS). The Company expects that the European Commission, based on this positive opinion of the COMP, will formally grant the orphan drug designation for the European Union (EU) by the end of July. Imetelstat has already been granted orphan drug designation by the United States Food and Drug Administration as a potential treatment for MDS.

"The positive opinion from the COMP acknowledges the compelling clinical data from our Phase 2 clinical trial that imetelstat has the potential to provide a clinically relevant advantage to lower risk MDS patients who are transfusion dependent and have failed to respond to treatment with erythropoiesis stimulating agents," said John A. Scarlett, Chairman and Chief Executive Officer. "Our ongoing IMerge Phase 3 clinical trial in lower risk MDS is being conducted at multiple sites around the world, and data from this trial are intended to support global regulatory filings."

To qualify for orphan drug designation in the EU, an investigational medicine must be intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient non-clinical or clinical data to suggest the investigational medicine may produce clinically relevant outcomes. EMA orphan drug designation provides companies with certain benefits and incentives, including clinical protocol assistance, differentiated evaluation procedures for Health Technology Assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees

and ten years of market exclusivity.

Ongoing IMerge Phase 3 Clinical Trial

The IMerge Phase 3 clinical trial is a double-blind, randomized, placebo-controlled clinical trial with registration intent. The trial is designed to enroll approximately 170 patients with lower risk transfusion dependent MDS who are relapsed or refractory to an ESA, have not received prior treatment with either a hypomethylating agent (HMA) or lenalidomide and who are non-del(5q). Geron expects to complete patient enrollment by the end of the first quarter of 2021 and top-line results to be available in the second half of 2022.

About Myelodysplastic Syndromes

MDS is a group of blood disorders in which the proliferation of malignant progenitor cells produces multiple malignant cell clones in the bone marrow resulting in disordered and ineffective production of the myeloid lineage, which includes red blood cells, white blood cells and platelets. Chronic anemia is the predominant clinical problem in patients who have lower risk MDS. Many of these patients become dependent on red blood cell transfusions due to low hemoglobin. Serial red blood cell transfusions can lead to elevated levels of iron in the blood and other tissues, which the body has no normal way to eliminate. Iron overload is a potentially dangerous condition. Studies in patients with MDS have shown that iron overload resulting from regular red blood cell transfusions is associated with lower quality of life, shorter overall survival and a higher risk of developing acute myeloid leukemia.

About Imetelstat

Imetelstat is a novel, first-in-class telomerase inhibitor exclusively owned by Geron and being developed in hematologic myeloid malignancies. Early clinical data suggest imetelstat may have disease-modifying activity through the apoptosis of malignant stem and progenitor cells, which allows potential recovery of normal hematopoiesis. Geron's imetelstat development program includes two ongoing or planned registration-enabling studies, IMerge, an ongoing Phase 2/3 clinical trial in lower risk myelodysplastic syndromes (MDS), and a planned Phase 3 clinical trial in refractory myelofibrosis (MF) expected to be open for patient screening and enrollment in the first quarter of 2021. Imetelstat has been granted Fast Track designation by the U.S. Food and Drug Administration for both the treatment of patients with non-del(5q) lower risk MDS who are refractory or resistant to an erythropoiesis-stimulating agent and for patients with Intermediate-2 or High-risk MF whose disease has relapsed after or is refractory to janus kinase (JAK) inhibitor treatment.

About Geron

Geron is a late-stage clinical biopharmaceutical company focused on the development and potential

commercialization of a first-in-class telomerase inhibitor, imetelstat, in hematologic myeloid malignancies. For more information about Geron, visit www.geron.com.

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) IMerge, Geron expects to complete enrollment in the first quarter of 2021 and have top-line results in the second half of 2022; (ii) that data from the IMerge Phase 3 clinical trial will support global regulatory filings; (iii) the period of potential market exclusivity in the EU with orphan drug designation; (iv) potential disease-modifying activity of imetelstat; (v) that the European Commission will grant the orphan drug designation; and (vi) 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 the evolving effects of the COVID-19 pandemic and resulting global economic and financial disruptions will materially and adversely impact Geron’s business and business prospects, its financial condition and the future of imetelstat; (b) whether Geron overcomes all of the potential delays and other adverse impacts caused by the evolving effects of the COVID-19 pandemic, and overcomes the clinical, safety, efficacy, technical, scientific, intellectual property, manufacturing and regulatory challenges in order to meet the expected timelines and planned milestones in (i) above; (c) whether regulatory authorities permit the further development of imetelstat on a timely basis, or at all, without any clinical holds; (d) whether imetelstat is demonstrated to be safe and efficacious in clinical trials; (e) whether any future efficacy or safety results may cause the benefit-risk profile of imetelstat to become unacceptable; (f) whether imetelstat actually demonstrates disease-modifying activity in patients; (g) Geron’s need to raise substantial capital in order to complete the development and commercialization of imetelstat, including to meet all of the expected timelines and planned milestones in (i) above; (h) whether Geron can accurately project or attain complete enrollment in the IMerge Phase 3 clinical trial, whether due to the evolving effects of the COVID-19 pandemic or otherwise; (i) whether EU regulatory authorities in the future decide that there are other drugs so similar to imetelstat that its orphan drug designation is not warranted and removed; and (j) whether there are failures or delays in manufacturing sufficient quantities of imetelstat or other clinical trial materials in a timely manner, whether due to the evolving effects of the COVID-19 pandemic or otherwise. 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 March 31, 2020. 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.

CONTACT:

Suzanne Messere
Investor and Media Relations
investor@geron.com
media@geron.com

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