First Clinical Data with MGTA-145 Show Single-Day Dosing and Collection of Robust Numbers of High-Quality Stem Cells in Healthy Volunteers

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 7, 2019-- **Magenta Therapeutics** (NASDAQ: MGTA), a clinical-stage biotechnology company developing novel medicines to bring the curative power of immune reset to more patients, today announced that new results from its MGTA-145 stem cell mobilization program were presented at the 61st Annual Meeting of the American Society of Hematology (ASH). These results, which were presented by John DiPersio, M.D., Ph.D., Professor of Medicine and Chief of the Oncology Division, Washington University School of Medicine, St. Louis, Missouri, showed that MGTA-145 in combination with plerixafor met all the endpoints in the study, and safely mobilized a large number of high-quality stem cells in a single day. The cells were then shown to engraft in a humanized mouse model.

Mobilized peripheral blood is used for the majority of the 65,000 stem cell transplants performed each year across the United States and Europe, but the current standard of care, G-CSF, requires at least five days of dosing and is associated with significant side effects, including bone pain that often requires narcotics. Further, patients with autoimmune diseases or sickle cell disease can have severe side effects with G-CSF, including potentially fatal complications.

Magenta is developing MGTA-145 as the new first-line standard of care for stem cell mobilization in a broad range of diseases, including autoimmune diseases and genetic diseases. MGTA-145, a CXCR2 agonist, works in combination with plerixafor, a CXCR4 antagonist, to harness the physiological mechanism of stem cell mobilization.

“The current standard of care for stem cell mobilization, G-CSF, requires five or more days of injections, which is difficult for patients, donors and the healthcare system. The novel mechanism of MGTA-145 may enable single-day dosing, mobilization and collection in all donors and patients, including those with sickle cell disease or autoimmune diseases, and positions it as a promising new first-line standard of care for all transplants as well as gene therapy,” said Dr. DiPersio.

“These initial results in a healthy volunteer setting validate the unique mechanism of action of MGTA-145 and its ability to mobilize sufficient hematopoietic stem cells for transplant in combination with plerixafor. We are particularly pleased to see the high percentage of CD34+CD90+ cells collected in healthy volunteers, as preclinical data suggest that these cells are correlated with engraftment. Our initial results support this, with rapid engraftment of the collected cells observed in humanized mouse models,” said John Davis, M.D., M.P.H., Chief Medical Officer, Magenta. “The unique mechanism, single-day dosing and collection of robust numbers of cells that engraft quickly highlight the potential utility for MGTA-145 across multiple diseases and hold promise for future development in allogenic donors as well as patients.”

Results from the MGTA-145 Phase 1 Study in Healthy Volunteers

Title: Rapid and Robust Mobilization of CD34+ HSCs without G-CSF Following Administration of MGTA-145 Alone and in Combination with Plerixafor (Abstract #1961)

Presenter: John DiPersio, M.D., Ph.D., Professor of Medicine and Chief of the Oncology Division, Washington University School of Medicine, St. Louis, Missouri

This study consists of four parts:

- In Part A, healthy volunteers were dosed with MGTA-145 (0.0075 – 0.3 mg/kg) or placebo.
- In Part B, subjects received a single dose of MGTA-145 (0.03 – 0.15 mg/kg) or placebo in combination with a single dose of plerixafor (0.24 mg/kg).
- In Part C, subjects received MGTA-145 or placebo plus plerixafor administered on day 1 and day 2.
- Part D is ongoing - subjects will receive a single dose of MGTA-145 (0.03 mg/kg) plus plerixafor followed by a single apheresis collection of multiple blood volumes.

Endpoints include safety and tolerability, pharmacokinetics, target engagement, and pharmacodynamic effects.

Results presented by Dr. DiPersio showed:

- MGTA-145 was safe and well-tolerated as a single agent and in combination with plerixafor and showed the expected target pharmacology.
- MGTA-145 engages CXCR2 on neutrophils to mobilize CD34+ cells into peripheral blood with limited neutrophil activation, which may minimize risk of vaso-occlusive crises in patients with sickle cell disease and immune flares in patients with autoimmune disease.
- Five of six subjects who received a single dose of the combination of MGTA-145 at the .03 dose level and plerixafor mobilized more than 20 CD34+ cells/microliter, the clinically accepted threshold for successful mobilization, in a single day.
- Subjects in Part C demonstrated reliable mobilization of $CD34^+$ cells on Day 2 with peak counts that were comparable to Day 1 mobilization yields, suggesting that two-day dosing and collection is feasible.
- Single-day dosing and apheresis collection in four subjects dosed to date in Part D yielded a median of 4.3 million CD34+ cells/kg (range: 2.7 to 5.3).

    - The clinically accepted threshold for a successful transplant is 2 million cells/kg.
- The median percentage of CD34+CD90+ cells was 33% (range: 10% to 41%), compared to approximately 10% with G-CSF-mobilized peripheral blood.
- Cells collected from the first two subjects dosed in Part D transplanted into humanized mice engrafted more rapidly and at a five fold higher level than G-CSF-mobilized peripheral blood.
- MGTA-145 in combination with plerixafor enables safe, same-day dosing, mobilization and collection of sufficient high-quality hematopoietic stem cells for transplant.

About Magenta Therapeutics

Magenta Therapeutics is a clinical-stage biotechnology company developing medicines to bring the curative power of immune system reset through stem cell transplant to more patients with autoimmune diseases, genetic diseases and blood cancers. Magenta is combining leadership in stem cell biology and biotherapeutics development with clinical and regulatory expertise, a unique business model and broad networks in the stem cell transplant world to revolutionize immune reset for more patients.

Magenta is based in Cambridge, Mass. For more information, please visit www.magentatx.com.

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