



Magenta Therapeutics Completes Dosing in Phase 1 MGTA-145 Trial, Demonstrating Rapid, Single-Day First Line Stem Cell Mobilization and Collection

February 24, 2020

- Updated data from the Phase 1 study in healthy subjects show that MGTA-145, in combination with plerixafor, met all primary and secondary endpoints –
- Median of 4.1 million CD34+ cells/kg collected in a single day from eight subjects –
- Magenta plans to initiate multiple Phase 2 studies in 2020, in both allogeneic and autologous transplant settings –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 24, 2020-- [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 the completion of dosing in its Phase 1 trial of stem cell mobilization therapy clinical candidate, MGTA-145, as well as updated clinical data from the trial at the Transplant and Cellular Therapy (TCT) Annual Meeting in Orlando, Florida.

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, blood cancers 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.

"Current options for stem cell mobilization for transplant are inefficient and cannot be used in all patients. MGTA-145 is a novel medicine developed as a completely new standard of care for first-line stem cell mobilization for all patients and donors," said John DiPersio, M.D., Ph.D., Professor of Medicine and Chief of the Oncology Division, Washington University School of Medicine, St. Louis, Missouri. "The data presented at TCT show safe and robust mobilization of sufficient cells for transplant in hours with MGTA-145 and plerixafor, compared to the typical five or more days of dosing required for G-CSF. Additionally, we saw rapid engraftment of the cells collected in the Phase 1 study in humanized mouse models."

Magenta has completed dosing in this Phase 1 trial, achieving all primary and secondary endpoints. The Company plans to move the MGTA-145 program into multiple Phase 2 studies in 2020. The Phase 2 studies will include both allogeneic and autologous transplant settings across multiple diseases and will evaluate mobilization and collection of high-quality cells and engraftment of the cells after transplant.

MGTA-145 Phase 1 Trial in Healthy Volunteers

These data provide further confirmation that MGTA-145, in combination with plerixafor, enables the same-day dosing and collection of sufficient hematopoietic stem cells (HSCs) for transplant.

Title: Phase 1 Clinical Study of MGTA-145 in Combination with Plerixafor Shows Rapid Single-Day Mobilization and Collection of CD34+ HSCs without G-CSF (Abstract #73)

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:

- Part A: Healthy volunteers were dosed with a single dose of MGTA-145 or placebo.
- Part B: Subjects received a single dose of MGTA-145 or placebo, in combination with plerixafor.
- Part C: Subjects received a single dose of MGTA-145 or placebo, in combination with plerixafor on two consecutive days.
- Part D: Eight subjects received a single dose of MGTA-145 (0.03 or 0.015 mg/kg) in combination with plerixafor, followed by a single apheresis collection of multiple blood volumes.

Endpoints include safety and tolerability, pharmacokinetics and pharmacodynamic effects.

- 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 adverse events typically seen with current standard of care.
- Ten of 12 subjects who received a single dose of the combination of MGTA-145 at the .03 dose level or .015 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 eight subjects across two dose ranges administered in Part D yielded a median of 4.1 million CD34+ cells/kg.

- The clinically accepted threshold for a successful transplant is 2 million cells/kg.
- The median percentage of CD34+CD90+ cells (functional stem cells) was 35%, compared to approximately 10% with standard of care.
- Stem cells collected from the first two subjects dosed in Part D transplanted into humanized mice engrafted more rapidly and at a 10-fold higher level than G-CSF-mobilized peripheral blood at a 12-week timepoint.
- MGTA-145 in combination with plerixafor enables safe, same-day dosing, mobilization and collection of sufficient functional hematopoietic stem cells for transplant.

About Magenta Therapeutics

Headquartered in Cambridge, Mass., Magenta Therapeutics is a clinical-stage biotechnology company developing novel medicines for patients with autoimmune diseases, blood cancers and genetic diseases. By creating a platform focused on critical areas of unmet need, Magenta Therapeutics is pioneering an integrated approach to allow more patients to receive one-time, curative therapies by making the process more effective, safer and easier.

Forward-Looking Statement

This press release may contain forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "will," "could," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "projects," "seeks," "endeavor," "potential," "continue" or the negative of such words or other similar expressions can be used to identify forward-looking statements. The express or implied forward-looking statements included in this press release are only predictions and are subject to a number of risks, uncertainties and assumptions, including, without limitation risks set forth under the caption "Risk Factors" in Magenta's Registration Statement on Form S-1, as updated by Magenta's most recent Quarterly Report on Form 10-Q and its other filings with the Securities and Exchange Commission. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this press release may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. You should not rely upon forward-looking statements as predictions of future events. Although Magenta believes that the expectations reflected in the forward-looking statements are reasonable, it cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. Moreover, except as required by law, neither Magenta nor any other person assumes responsibility for the accuracy and completeness of the forward-looking statements included in this press release. Any forward-looking statement included in this press release speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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