



Magenta Therapeutics Announces Updated Phase 2 Data on MGTA-456 Cell Therapy, Demonstrating Continued Durability in Inherited Metabolic Disorders

February 20, 2020

-- Additional data from Phase 2 study show that MGTA-456 demonstrates clinically meaningful durable benefits for patients with inherited metabolic disorders one year following treatment --

-- Magenta intends to complete enrollment in Phase 2 in 2020 and continue dialogue with the FDA under the RMAT designation on design of a registration-enabling study, and to have discussions with the European Medicines Agency for development in Europe as well --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 20, 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 updated clinical data from Phase 2 trials of its cell therapy, MGTA-456, at the Transplant and Cellular Therapy (TCT) Annual Meeting in Orlando, Florida. New results from Magenta's MGTA-117 conditioning program and MGTA-145 stem cell mobilization program will be presented at TCT later this week.

MGTA-456 is a cell therapy designed to provide a high dose of hematopoietic stem cells (HSCs) that are well matched to the patient to enable safe immune and blood system rebuild in IMD patients and remission in patients with blood cancers. Magenta is currently developing MGTA-456 in an ongoing Phase 2 study in patients with inherited metabolic disorders (IMD), including cerebral adrenoleukodystrophy (cALD), mucopolysaccharidosis type IH (MPS I, or Hurler syndrome), metachromatic leukodystrophy (MLD) or globoid cell leukodystrophy (GLD, or Krabbe disease). These are rare, rapidly progressive neurologic disorders that are fatal when left untreated. Investigators at the University of Minnesota are also studying the cryopreserved formulation of MGTA-456 in a Phase 2 clinical trial in patients with high-risk blood cancers.

"The clinical demonstration of rapid and durable resolution of disease in patients with inherited metabolic disorders is very compelling; it's particularly encouraging as these results are not seen with currently available treatments, nor with gene therapies under investigation," said John Davis, MD, MPH, Chief Medical Officer, Magenta Therapeutics. "Data from the University of Minnesota study in blood cancers add to the body of safety and engraftment data for MGTA-456, and, importantly, validate the introduction of cryopreserved 456 product into the Phase 2 study of inherited metabolic disorders, crucial for the establishment of multi-center trials, as well as eventual global patient access."

Magenta intends to complete enrollment in the Phase 2 in 2020 and continue dialogue with the FDA under the RMAT designation on design of a registration-enabling study, and to have discussions with the European Medicines Agency for development in Europe.

Updated Results from Ongoing MGTA-456 Phase 2 Study in Inherited Metabolic Disease

Title: MGTA-456 Cell Therapy in Inherited Metabolic Disease Yields Rapid and Durable Long-Term Improvement of Disease-Specific Outcomes in a Phase 2 Trial (Abstract #20)

Presenter: Paul J. Orchard, MD, University of Minnesota Medical Center

Results:

- Treatment with MGTA-456 in patients with inherited metabolic disorders showed early, robust engraftment and immune reconstitution.
- Patients received a median CD34⁺ cell dose of 110×10^6 cells/kg and TNC dose of 26×10^7 cells/kg with a median duration of neutropenia of one day (range 0-9), compared to a median of eight days for historical controls.
- Myeloid chimerism was $\geq 98\%$ donor in evaluable patients by day +14 and immune reconstitution of CD4 and CD8 T-cell subsets were comparable or better than historical controls.
- Two steroid-responsive episodes of skin-only aGvHD were observed and no cGvHD has been reported.

Key results in patients with cALD:

- Patients showed resolution of gadolinium enhancement on MRI, an indicator of brain inflammation, by one month post-treatment, and the resolution persisted throughout the follow-up period. Sustained resolution of gadolinium enhancement is correlated with long-term disease benefit in patients with cALD.
- The Loes score, a method for quantifying the severity of brain abnormalities and atrophy found on MRI, remained stable over the follow-up period, consistent with a halt in disease progression.
- The NFS remained stable in both patients over one year, suggesting a durable halt in disease progression.

Key results in patients with MPS I / Hurler Syndrome:

- Patients with MPS I / Hurler syndrome showed normalized levels of blood a-L-iduronidase by Day +42 and had decreased levels of Hurler-specific urine. glycosaminoglycans, the toxic metabolites implicated in disease at a >42-day timepoint.

In a separate presentation today, John Wagner, M.D., University of Minnesota, presented results from a Phase 2 trial of MGTA-456 in patients with high-risk hematologic malignancies. All patients treated to date in this Phase 2 trial successfully engrafted, with rapid neutrophil recovery.

Additionally, Kevin Goncalves, Ph.D., Magenta Therapeutics, presented preclinical data demonstrating rapid and durable resolution of CNS, peripheral and skeletal abnormalities associated with IMDs in a Hurler mouse model following a high dose of CD34+ stem cells. This supports the hypothesis that a higher dose of CD34+ cells, such as MGTA-456, is linked to earlier engraftment and disease impact, and suggests that MGTA-456 may have impact on the disease in the periphery and skeleton.

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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