



## Magenta Therapeutics Presents Updated Phase 2 Clinical Data on MGTA-456 Cell Therapy at American Academy of Neurology Annual Meeting

May 9, 2019

*-- Six-month follow-up on patients with cerebral adrenoleukodystrophy (cALD) shows stable neurological function scores and early and persistent resolution of brain inflammation on MRI --*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 9, 2019-- [Magenta Therapeutics](#) (NASDAQ: MGTA), a clinical-stage biotechnology company developing novel medicines to bring the curative power of stem cell transplant to more patients, today announced that the Company presented Phase 2 clinical data on its cell therapy, MGTA-456, at the annual meeting of the American Academy of Neurology in Philadelphia, Pennsylvania.

MGTA-456 is a cell therapy designed to provide a high dose of hematopoietic stem cells that are well-matched to the patient. The Company plans to enroll 12 patients in the ongoing Phase 2 study in inherited metabolic disorders, which include cALD, Hurler syndrome, metachromatic leukodystrophy and globoid cell leukodystrophy. The primary endpoint of the study is neutrophil engraftment after transplantation. The study is also collecting both short- and long-term disease-specific outcomes. Data from the first five evaluable patients treated in this study were highlighted in a poster presented by Ashish Gupta, MBBS, M.P.H., Assistant Professor, Department of Pediatrics and Division of Blood and Marrow Transplantation, University of Minnesota.

In a separate oral presentation today, Kevin Goncalves, Ph.D., Magenta Therapeutics, will highlight preclinical data demonstrating that the high stem cell dose in MGTA-456 accelerates and improves engraftment of human microglia in the brains of transplanted mice.

"We are very pleased to see signs of durable disease benefit in patients with cALD," said John Davis, M.D., M.P.H., Chief Medical Officer, Magenta Therapeutics. "cALD is a rapidly progressive disease, and patients whose disease progresses quickly typically have poor long-term outcomes. The stable neurological function score and persistent decrease in brain inflammation in these two patients suggest that we have halted the inflammatory process associated with the disease which may provide long-term benefits. We look forward to providing an additional update from the study before the end of the year."

### **Patients with Inherited Metabolic Disorders (IMDs) transplanted with MGTA-456, a CD34+ Expanded Cell Therapy Product, Show Rapid Engraftment in Preliminary Phase 2 Trial Results**

Key results in patients with cALD:

- Both patients had stable neurological function scores, which remained unchanged between baseline and six months post-transplant, suggesting progress of the disease has been arrested.
- The Loes score, a method for quantifying the severity of brain abnormalities and atrophy found on MRI, also remained stable in both patients after six months.
- Both patients showed resolution of gadolinium enhancement on MRI, an indicator of brain inflammation, by one month post-transplant, and the resolution persisted at six months.
  - Durable resolution of gadolinium enhancement is correlated with long-term disease benefit in patients with cALD.

Key results in patients with Hurler Syndrome:

- As previously reported, all three patients with Hurler syndrome achieved normal levels of blood leukocyte IDUA enzyme, the enzyme that is deficient in untreated patients with Hurler syndrome, by Day 42 post-transplant. This suggests that transplant with MGTA-456 is affecting the disease process in these patients.
  - Normalization of blood leukocyte IDUA enzyme after transplant has been significantly associated with improvement in disease.
  - Patients showed a marked decline in urine total glycosaminoglycan (GAG), the toxic metabolites implicated in disease, after transplant.
  - Both of these findings are correlated with improved long-term disease outcomes.

Overall results:

- All five patients met the primary endpoint of neutrophil engraftment
- MGTA-456 was well tolerated

### **Forward-Looking Statement**

This press release contains 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,

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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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