Magenta Therapeutics Announces FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to MGTA-456 for the Treatment of Inherited Metabolic Disorders

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 4, 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 U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advance Therapy (RMAT) designation for MGTA-456, a one-time cell therapy for the treatment of multiple inherited metabolic disorders.

“This RMAT designation was based on the encouraging clinical data we have presented thus far, and it is an important milestone that recognizes the transformative, life-saving potential of MGTA-456 for children suffering from inherited metabolic disorders,” said John Davis, M.D., M.P.H., Chief Medical Officer, Magenta. “We look forward to collaborating closely with the FDA as we seek to rapidly advance MGTA-456 through the ongoing Phase 2 study and into potential pivotal studies in 2020.”

MGTA-456 is a cell therapy designed to halt the progress of inherited metabolic disorders by providing a high dose of stem cells that regenerate a well-matched new immune system in the patient. Magenta is developing MGTA-456 as a medicine for multiple diseases. The company is currently studying MGTA-456 in a Phase 2 clinical trial in patients older than 6 months of age with Hurler syndrome, cerebral adrenoleukodystrophy (cALD), metachromatic leukodystrophy (MLD) or globoid cell leukodystrophy (GLD) at four centers of excellence: Cincinnati Children’s Hospital, Duke University, Emory University and the University of Minnesota.

Previously presented results from the first five evaluable patients treated in the study followed to 6 months showed rapid and persistent signs of disease benefit, which are not consistently seen with other investigational therapies in these diseases:

- Patients with Hurler syndrome showed normalization of previously deficient enzyme levels in the blood as early as 42 days following treatment and continued decrease in toxic metabolites 6 months following treatment.
- Patients with cALD showed resolution of inflammation in the brain on MRI as early as 28 days following treatment, and stable neurological function scores, 6 months post-treatment, 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 at 6 months.
- All five evaluable patients achieved the FDA-agreed primary endpoint of neutrophil engraftment.
- Magenta expects to present updated data from the study before the end of 2019.

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the development and approval processes for promising pipeline products, including cell therapies. An investigational drug is eligible for RMAT designation if it is intended to treat, modify, reverse, or cure a serious or life-threatening disease; and preliminary clinical evidence indicates that the therapy has the potential to address unmet medical needs for that disease. Advantages of the RMAT designation include all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with FDA that may be used to discuss potential surrogate or intermediate endpoints to support accelerated approval.

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: uncertainties inherent in clinical studies and in the availability and timing of data from ongoing clinical studies; whether interim results from a clinical trial will be predictive of the final results of the trial; whether results from preclinical studies or earlier clinical studies will be predictive of the results of future trials; the expected timing of submissions for regulatory approval or review by governmental authorities, including review under accelerated approval processes; orphan drug designation eligibility; regulatory approvals to conduct trials or to market products; and other 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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