Magenta Therapeutics Provides Portfolio Update: Stem Cell Mobilization and Conditioning Programs
Prioritized and Advancing Toward Clinical Milestones

June 11, 2020

— MGTA-145 first-line mobilization program to move into Phase 2 studies in autologous and allogeneic patients in 2020, including allogeneic study in newly announced collaboration with National Marrow Donor Program® (NMDP)/Be The Match® —

— MGTA-117 antibody-drug conjugate (ADC)-based conditioning program on track for initial clinical data in 2021; research and clinical collaboration with AVROBIO announced May 2020 to jointly evaluate potential usage in gene therapy settings —

— Company has made strategic decision to discontinue enrollment in Phase 2 study of MGTA-456 in inherited metabolic disorders (IMDs) and prioritize resources toward stem cell mobilization and conditioning programs —

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 11, 2020-- 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 provided a portfolio update, highlighting progress and recent updates across its most advanced programs.

“Magenta has multiple high-priority medicines in our portfolio, including the mobilization and targeted conditioning programs, which have the potential to benefit many patients with genetic diseases, blood cancers and autoimmune diseases. We believe that these new medicines will provide patients with life-saving treatments and we are focusing our resources to move these programs forward as efficiently as possible,” said Jason Gardner, D.Phil., President and Chief Executive Officer, Magenta Therapeutics. “We have made the strategic decision based on patient benefit, emerging new results, collaborations and regulatory input to prioritize these programs as they advance through clinical development. As part of this strategic decision, we will discontinue enrollment in the Phase 2 study of MGTA-456 cell therapy in patients with inherited metabolic disorders. We thank the brave patients and their families who participated in the trial, as well as the dedicated investigators and team members at the clinical trial sites.”

**Portfolio Updates**

- **MGTA-145 first-line stem cell mobilization therapy**: Magenta is developing MGTA-145 for a broad range of diseases, including autoimmune diseases, blood cancers and genetic diseases. The recently completed Phase 1 study in healthy donors showed that MGTA-145, in combination with plerixafor, enables safe, same-day dosing, mobilization and collection of sufficient functional hematopoietic stem cells for transplant.
  - Based on the results of the Phase 1 study and a productive end of Phase 1 meeting with the U.S. Food and Drug Administration (FDA), Magenta intends to initiate multiple Phase 2 trials of MGTA-145 to include both allogeneic and autologous transplant settings, with up to 150,000 patients eligible annually in the US and Europe. There is potential for Magenta to generate initial Phase 2 data on MGTA-145 in 2020.
  - In May 2020, the FDA’s Office of Orphan Products and Development granted Orphan Drug Designation to MGTA-145 for the mobilization of hematopoietic stem cells to the peripheral blood for collection and subsequent transplant.
  - Magenta announced a clinical collaboration agreement with National Marrow Donor Program® (NMDP)/Be The Match® to evaluate MGTA-145 in a Phase 2 study for single-day mobilization of healthy donor stem cells for patients undergoing allogeneic transplant. NMDP/Be The Match is the leading stem cell transplant organization in the United States and manages the largest stem cell donor registry in the world.

- **Conditioning Portfolio — MGTA-117 lead clinical candidate for ADC-based conditioning for stem cell transplant and gene therapy**: The Company’s most advanced conditioning program is on track to complete IND-enabling toxicology studies and progress GMP manufacturing in 2020, and Magenta expects to deliver initial clinical data in 2021.
  - In May 2020, Magenta announced a research and clinical collaboration with AVROBIO to evaluate the potential utility of MGTA-117 for conditioning patients with lysosomal storage disorders before they receive one of AVROBIO’s investigational lentiviral gene therapies.

- **Conditioning Portfolio – CD45-ADC for immune reset**: Magenta presented preclinical data on its CD45-ADC program at the European League Against Rheumatism (EULAR) annual meeting last week demonstrating that a single dose of CD45-ADC removed disease-causing reactive T cells, enabling successful immune reset to halt disease progression and was well tolerated in three models of autoimmune disease: multiple sclerosis, systemic sclerosis and inflammatory arthritis. Magenta has identified a lead antibody for this program, and IND-enabling work on CD45-ADC is progressing in 2020.

- **MGTA-456 cell therapy**: Magenta has made the strategic decision to discontinue enrollment in the Phase 2 study in IMDs. This decision was the result of several factors: enrollment challenges common to rare disease populations, particularly during the COVID-19 pandemic; a growing understanding in the field of the current challenges of allogeneic stem cell transplant in patients with non-malignant diseases, such as IMDs; and feedback from the FDA on endpoints and clinical trial design for registration. Enrollment in the Phase 2 investigator-initiated trial in patients with blood cancers is expected to be completed soon. The Company will use these data to inform a decision regarding future program development in blood cancers.
Magenta will participate in a fireside chat at the 41st annual Goldman Sachs Healthcare Conference today at 8:50 a.m. ET. A live webcast of the fireside chat can be accessed on the Magenta Therapeutics website at https://investor.magentatx.com/events-and-presentations. The webcast replay will be available for 90 days following the event.

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, including express or implied statements regarding Magenta’s future expectations, plans and prospects, including, without limitation, statements regarding expectations and plans for presenting pre-clinical and clinical data, the anticipated timing of our clinical trials and regulatory filings, the development of our product candidates and advancement of our preclinical programs, as well as other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “project,” “should,” “target,” “will” or “would” and similar expressions that constitute forward-looking statements under the Private Securities Litigation Reform Act of 1995. 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 preclinical and clinical studies and in the availability and timing of data from ongoing preclinical and 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; whether Magenta’s cash resources will be sufficient to fund Magenta’s foreseeable and unforeseeable operating expenses and capital expenditure requirements; risks, uncertainties and assumptions regarding the impact of the COVID-19 pandemic on Magenta’s business, operations, strategy, goals and anticipated timelines; the timing, progress and success of the Magenta’s collaborations with NMDP/Be the Match and AVROBIO; and other risks concerning Magenta’s programs and operations are described in additional detail in its Annual Report on Form 10-K filed on March 3, 2020, as updated by its most recent Quarterly Report on Form 10-Q and its other filings made with the Securities and Exchange Commission from time to time. Although Magenta’s forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Magenta. As a result, you are cautioned not to rely on these forward-looking statements. Any forward-looking statement made in this press release speaks only as of the date on which it is made. Magenta undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future developments or otherwise.

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