Magenta Therapeutics Advances Conditioning Platform and Clinical Programs, Highlights Recent Milestones and 2020 Goals

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- *New MGTA-117 ADC clinical candidate for conditioning demonstrates broad therapeutic index; advancing MGTA-117 to generate initial patient clinical data in 2021*
- *MGTA-145 first-line stem cell mobilization agent on track to complete Phase 1 study and move into multiple Phase 2 studies in 2020*
- *Completion of enrollment in Phase 2 study of MGTA-456 cell therapy in IMDs expected in 2020*
- *Eight abstracts from across the pipeline accepted for presentation at Transplant and Cellular Therapy Annual Meeting, including four oral presentations, and Best Oral Abstract award*
- *Ended 2019 with approximately $146 million in cash and cash equivalents with runway into 4Q21*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 13, 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 highlighted recent progress across several programs and outlined goals for 2020. These updates will be discussed during a webcast presentation at the 38th annual J.P. Morgan Healthcare Conference on Wednesday, January 15th at 11:30 a.m. PT (2:30 p.m. ET).

“In 2019 we generated landmark data from our ADC-based targeted patient preparation platform, which is delivering a new class of antibody-drug conjugates (ADCs) that have the power to bring one-time treatment to more patients with autoimmune diseases, blood cancers and genetic diseases. We also presented clinical data for our first-line stem cell mobilization program, MGTA-145, which we are developing as the new standard of care for stem cell mobilization with the potential to benefit all of the transplant-eligible patients each year,” said Jason Gardner, D. Phil., President and Chief Executive Officer, Magenta. “As we begin 2020, we are particularly excited to unveil our MGTA-117 clinical candidate for targeted patient preparation for stem cell transplant or gene therapy. New results announced today highlight the potency, safety and broad therapeutic index of MGTA-117, well above that of currently approved ADCs. We believe that MGTA-117 is the optimal agent for depleting stem cells to enable safe immune reset. We look forward to moving this program into the clinic with initial clinical data expected in 2021.”

Targeted Patient Preparation Programs

Current methods to condition patients before transplant and gene therapy are dependent on toxic, non-specific chemotherapy or radiation. These pre-transplant treatments are associated with significant side effects, including infertility, cancer, organ damage and death. Magenta is developing targeted, disease-modifying ADCs that are designed to precisely and rapidly remove the disease-causing cells in the body and enable immune system reset without the need for chemotherapy or radiation.

CD117-ADC Recent Progress

Data presented at the American Society of Hematology (ASH) annual meeting in December 2019, showed the first-ever successful transplant of gene-modified cells in non-human primates using a CD117-targeted, single-agent ADC from Magenta, without the use of chemotherapy or radiation. These unprecedented results validate and advance Magenta’s conditioning platform.

Building on this work, Magenta’s new clinical candidate, MGTA-117, is a CD117 antibody conjugated to amanitin. Results published today in an abstract for the Transplant and Cellular Therapy annual meeting show that MGTA-117 potently depleted stem and progenitor cells and demonstrated a wide tolerability: potency ratio of 30 fold (therapeutic index; typical range for approved ADCs at this stage is two to six fold). This program is advancing to the clinic and further validates Magenta’s antibody drug conjugate-based conditioning platform. MGTA-117 was developed under a partnership with Heidelberg Pharma that grants Magenta exclusive worldwide development and marketing rights for ADCs using an amanitin payload and targeting CD117.

MGTA-117 in 2020

Magenta is scaling up manufacturing of MGTA-117 and completing IND-enabling studies in 2020. The Company intends to move this new product candidate into the clinic with initial clinical data expected in 2021.

CD45-ADC Recent Progress

Current standard treatment for patients with multiple sclerosis involves years of chronic dosing of medications that do not halt the progression of the disease. For patients with systemic sclerosis, a potentially fatal autoimmune disease, there are no approved therapies. Immune reset through stem cell transplant has demonstrated durable remissions in thousands of patients with autoimmune diseases such as multiple sclerosis and systemic sclerosis, and it is recommended by the European League Against Rheumatism (EULAR) in treatment guidelines for systemic sclerosis. The immune reset process involves two main steps: removing the disease-causing cells and replacing them with healthy cells to rebuild the immune system to a healthy state.

Magenta is developing targeted ADCs designed to precisely remove the disease-causing cells in the body without the need for chemotherapy or radiation. Magenta’s CD45-ADC program targets CD45, a protein expressed on immune cells and stem cells and is designed to remove the cells that cause autoimmune diseases in order to enable curative immune reset.

Data presented at the American College of Rheumatology (ACR) meeting in November 2019 showed that a single dose of CD45-ADC removed disease-causing reactive T cells, enabled successful immune reset and rebuild of the immune system and was well tolerated in three models of autoimmune disease, including the EAE model, the most reliable murine model of multiple sclerosis. Further, a single dose of CD45-ADC significantly reduced disease incidence and delayed disease onset in this model that has successfully provided preclinical proof of concept for many clinically validated standard-of-care therapies.

CD45-ADC in 2020

Magenta has identified a lead antibody and has progressed this program into IND-enabling studies, which the Company plans to further advance in 2020.

MGTA-145 First-Line Stem Cell Mobilization Therapy

MGTA-145 Recent Progress

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.

Magenta is currently studying MGTA-145 and plerixafor in a Phase 1 study in healthy volunteers. Data from the Phase 1 study presented at the ASH annual meeting in December 2019 showed that MGTA-145 in combination with plerixafor successfully enables safe, same-day dosing, mobilization and collection of sufficient high-quality hematopoietic stem cells for transplant. Further, when cells collected from the first two apheresis subjects were transplanted into humanized mice, the cells engrafted more rapidly and at a five-fold higher level than cells from G-CSF-mobilized peripheral blood.

MGTA-145 in 2020

Magenta intends to complete the Phase 1 study and move this program into multiple Phase 2 studies in patients in 2020. The Phase 2 studies will include both allogeneic and autologous transplant settings and will evaluate mobilization and collection of high-quality cells and engraftment of the cells after transplant.

MGTA-456 Cell Therapy

MGTA-456 Recent Progress

MGTA-456 is a cell therapy designed to provide a high dose of stem cells that are well matched to the patient to enable safe immune and blood system rebuild and durable remissions in patients with blood cancers. In September, the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation for MGTA-456 for the treatment of multiple inherited metabolic disorders.

Magenta is currently studying MGTA-456 in a Phase 2 study in patients with inherited metabolic disorders, including cerebral adrenoleukodystrophy (cALD) and Hurler syndrome. These are rare, rapidly progressive neurologic disorders that are fatal when left untreated. Results in the first two evaluable patients with cALD updated in December 2019 showed early and durable resolution of the disease at 12 months' follow-up. The Loes score and NFS score, which measure progress of the disease, remained stable, suggesting that progress of the disease has been halted in these patients. The early and durable resolution of disease with MGTA-456 is not consistently seen with other therapies, including standard stem cell transplant, gene therapy or enzyme replacement therapy.

MGTA-456 in 2020

Magenta intends to complete enrollment in the Phase 2 in 2020 and continue dialogue with the FDA under the RMAT designation, and to discuss with the European Medicines Agency (EMA) for development in Europe

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 Annual Report on Form 10-K, as updated by Magenta's most recent Quarterly Reports 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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