Magenta Therapeutics to Present New Clinical and Preclinical Data at American Society of Hematology (ASH) Annual Meeting

November 6, 2019

-- First clinical data from Phase 1 study of MGTA-145 show successful mobilization of target number of stem cells in 11 of 12 healthy volunteers within hours; updated data including apheresis collection to be presented at ASH --

-- Single dose of CD117-ADC demonstrates first-ever successful gene therapy transplant in primates without chemotherapy conditioning, in collaboration with NIH --

-- Additional presentations from across the pipeline will showcase progress in autoimmune diseases, blood cancers and genetic diseases --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 6, 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 new data from across its pipeline will be presented at the 61st Annual Meeting of the American Society of Hematology (ASH).

“The breadth of new results we are presenting at ASH illustrates our progress toward enabling patients with autoimmune diseases, blood cancers and genetic diseases to benefit from curative stem cell transplant. In today’s abstracts we are particularly excited to share the first clinical data from our MGTA-145 program, which we are developing as a first-line stem cell mobilization drug. The data in the abstract show that MGTA-145 in combination with plerixafor was well-tolerated and successfully mobilized the target stem cell dose in 11 of 12 subjects within hours of administration. At ASH we anticipate sharing initial data from mobilization and collection of the target number of cells in subjects in a single-day procedure for the first time,” said Jason Gardner, D.Phil., Chief Executive Officer and President, Magenta Therapeutics. “We are also pleased to show groundbreaking data from our most advanced patient preparation program, CD117-ADC, in today’s abstract. These data, which will be shared in an oral presentation at ASH by Dr. John Tisdale of the National Institutes of Health, show the first-ever successful transplant in non-human primates using a targeted, single-agent antibody-drug conjugate, without the use of chemotherapy or radiation. These data provide additional validation for both our CD117-ADC program as well as our entire ADC-based patient preparation portfolio.”

First Data from MGTA-145 Clinical Program

Title: Rapid and Robust Mobilization of CD34+ HSCs without G-CSF Following Administration of MGTA-145 Alone and in Combination with Plerixafor (Abstract #1961)

Presenter: John DiPersio, M.D., Ph.D., Professor of Medicine and Chief of the Oncology Division, Washington University School of Medicine, St. Louis, Missouri

Date and Time: Saturday, December 7th, 5:30 to 7:30 p.m.

Mobilized peripheral blood is used for the majority of the 65,000 stem cell transplants performed each year across the United States and Europe, but the current standard of care, G-CSF, requires at least five days of dosing and is associated with significant side effects, including bone pain that often requires narcotics. Further, patients with autoimmune diseases or sickle cell disease can have severe side effects with G-CSF, including potentially fatal complications.

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 and genetic diseases. MGTA-145 works in combination with plerixafor to harness the physiological mechanism of stem cell mobilization. The MGTA-145-based combination has the potential to reliably mobilize robust numbers of high-quality stem cells with single-day dosing and collection. Importantly, the MGTA-145-based combination also avoids the need for G-CSF.

The ASH abstract includes early results from the Phase 1 study, as of the late July data cut-off. MGTA-145 was safe and well-tolerated as a single agent and in combination with plerixafor, and showed the expected target pharmacology. Eleven of the 12 subjects who received the combination of MGTA-145 and plerixafor mobilized more than 20 CD34+ cells/microliter, the clinically accepted threshold for successful mobilization. This number is considered predictive of collection of at least 2 million CD34+ stem cells/kg through apheresis, the threshold for cell dose for a successful transplant. Magenta anticipates that initial data from additional subjects and apheresis collection results will be presented at ASH.

Data from CD117-ADC Patient Preparation Program

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 the only company developing targeted antibody-drug conjugates (ADCs) designed to precisely remove the disease-causing cells in the body, without the need for chemotherapy or radiation.

Title: A Single Dose of CD117 Antibody Drug Conjugate Enables Autologous Gene-Modified Hematopoietic Stem Cell Transplant (Gene Therapy) in Nonhuman Primates (Abstract #610)

Presenter: John Tisdale, M.D., Director, Molecular and Clinical Hematology Section, National Institutes of Health, Bethesda, Md.

Date and Time: Monday, December 9th, 7:45 a.m.

Magenta’s most advanced patient preparation program, CD117-ADC, targets CD117, a protein expressed on hematopoietic stem cells. CD117-ADC is designed to remove the genetically mutated cells that cause certain genetic diseases, such as sickle cell disease, enabling curative stem cell transplant or gene therapy.

Data in the ASH abstract showed that a single dose of CD117-ADC enabled successful transplant of gene-modified stem cells for gene therapy in
non-human primates without the need for chemotherapy or radiation. CD117-ADC removed stem cells in the non-human primates, with a favorable safety profile. The ADC spared the immune system and was cleared rapidly as designed. In a rhesus model of gene therapy, a single dose of the ADC enabled engraftment of stem cells modified with the β-globin gene, the gene that causes sickle cell disease and β-thalassemia when mutated. These proof-of-concept studies validate the use of CD117-ADC for targeted stem cell depletion prior to transplant and support its use as a new conditioning agent for gene therapy and stem cell transplant without toxic chemotherapy or radiation.

Additional Posters and Presentations

**Title:** A Novel Targeted Approach to Achieve Immune System Reset: Magenta's CD45-Targeted Antibody-Drug Conjugates Enable Autologous HSCT, Ameliorate Disease in Autoimmune Models, Potently Kill Human Immune Cells from Normal Donors and MS patients, and Achieve Immune Depletion in Non-human Primates (NHP) (Abstract #3208)

**Presenter:** Geoff Gillard, Ph.D., Magenta Therapeutics, Cambridge, Mass.

**Date and Time:** Sunday, December 8th, 6:00 to 8:00 p.m.

**Title:** MGTA-456, an Aryl Hydrocarbon Receptor (AHR) Antagonist Based Expansion of CD34+ Hematopoietic Stem Cells (HSC), Permits Selection of Better HLA Matched Cord Blood Units (CBUs) and Promotes Faster Neutrophil Recovery and Uniform Engraftment with Potentially Less Acute Graft-vs-Host Disease (GVHD) (Abstract #804)

**Presenter:** John Wagner, M.D., Executive Medical Director, Bone Marrow Transplant Program, University of Minnesota, Minneapolis, Minn.

**Date and Time:** Monday, December 9th, 4:00 p.m.

**Title:** High Dose Hematopoietic Stem Cell Transplantation Leads to Rapid Hematopoietic and Microglia Recovery and Disease Correction in a Mouse Model of Hurler Syndrome (Abstract #4424)

**Presenter:** Kevin Goncalves, Ph.D., Magenta Therapeutics, Cambridge, Mass.

**Date and Time:** Monday, December 9th, 6:00 p.m. to 8:00 p.m.

**Title:** Ex Vivo Hematopoietic Stem Cell Expansion with E478 Increases the Rate of CRISPR-mediated Homology Directed Repair of the Beta Globin Gene in Human Stem Cells by >10-fold and Improves In Vivo Engraftment by >120-fold (Abstract #4634)

**Presenter:** Kevin Goncalves, Ph.D., Magenta Therapeutics, Cambridge, Mass.

**Date and Time:** Monday, December 9th, 6:00 p.m. to 8:00 p.m.

ASH Investor Event and Webcast to Review ASH Data and Updated MGTA-456 Phase 2 Clinical Data

Magenta will host a live webcast of an investor and analyst event at 8:30 p.m. ET on Saturday, December 7th, during the ASH Annual Meeting. During this event, Magenta will review data presented at the ASH meeting and present updated data from the ongoing Phase 2 clinical trial of MGTA-456 in inherited metabolic disorders.

To access the webcast, please visit the "News & Events" page within the Investors & Media section of the Magenta website at [www.magentatx.com](http://www.magentatx.com). A replay of the webcast will be available on the Magenta website for 90 days following the call.

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.


Source: Magenta Therapeutics

Manisha Pai, Vice President, Communications & Investor Relations
617-510-9193
mpai@magentatx.com