Magenta Therapeutics Announces Data Presentations Related to its Mobilization and Conditioning Programs at the 2021 American Society of Hematology (ASH) Annual Meeting

November 4, 2021

-- Positive topline clinical data from MGTA-145 investigator-initiated Phase 2 clinical trial in multiple myeloma --

-- Successful conditioning with monotherapy CD117 antibody drug conjugate in a primate model of transplant for gene therapy of sickle cell disease --

-- Successful conditioning with CD117 antibody drug conjugate in combination with lymphodepleting antibodies leading to effective allogeneic hematopoietic stem cell transplant in a murine model of acute myeloid leukemia --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 4, 2021-- **Magenta Therapeutics** (Nasdaq: MGTA), a clinical-stage biotechnology company developing novel medicines designed to bring the curative power of stem cell transplants to more patients, today announced positive top-line results from an investigator-initiated Phase 2 clinical trial of MGTA-145 stem cell mobilization in multiple myeloma. The data were accepted for a poster presentation at the 2021 American Society of Hematology (ASH) Annual Meeting, to be held in Atlanta and virtually from December 11-14, 2021. Oral and poster presentations of preclinical data related to the company's CD117 targeted conditioning program will also be made at the ASH Annual Meeting.

"We have made significant progress with our mobilization and targeted conditioning programs and we look forward to the presentation of the data that have been generated to support both programs," said Jason Gardner, D.Phil., President and Chief Executive Officer, Magenta Therapeutics.

Stem Cell Mobilization and Collection Program (MGTA-145)

Poster Presentation Highlighting Investigator-Initiated Phase 2 Clinical Data of MGTA-145 Stem Cell Mobilization in Multiple Myeloma:

Title: MGTA-145 + Plerixafor Provides G-CSF-Free Rapid and Reliable Hematopoietic Stem Cell Mobilization for Autologous Stem Cell Transplant in Patients with Multiple Myeloma: A Phase 2 Study (Poster #3888)

Date and Time to View Poster Presentation: Monday, December 13, 2021, 6:00pm – 8:00pm ET

Trial Design

Surbhi Sidana, M.D., Assistant Professor of Medicine in the Division of Blood and Marrow Transplantation and Cellular Therapy at Stanford University School of Medicine led this investigator-initiated, Phase 2 open-label clinical trial. The trial evaluated the ability of MGTA-145, in combination with plerixafor, to mobilize stem cells for autologous stem cell transplantation in patients with multiple myeloma. This trial had broad inclusion criteria and included the transplant-eligible population of patients with multiple myeloma who may have a variety of risk factors for mobilization.

Topline Clinical Data

- *Primary and Secondary Endpoints.* The trial has fully enrolled 25 patients with multiple myeloma. 88% of patients (22/25) treated with MGTA-145 plus plerixafor met the primary endpoint of mobilization and collection of 2 million CD34+ stem cells per kg in up to two days of same-day mobilization and apheresis. 68% of patients (17/25) achieved the primary endpoint in a single day of dosing and collection. Three patients who did not meet the primary endpoint successfully collected hematopoietic stem cells (HSCs) with subsequent G-CSF plus plerixafor dosing and 2-3 apheresis sessions. Secondary endpoints of 4 million and 6 million CD34+ stem cells per kg in up to two days were met in 68% (17/25) and 40% (10/25) patients, respectively.
- *Days of Stem Cell Collection.* The median number of 5.0 million CD34+ stem cells per kg were collected cumulatively over one or two days of dosing and stem cell collection. In contrast, current standard of care with G-CSF-based regimens require a minimum of five days of dosing prior to initiating stem cell collection over one to four days.
- *Safety Profile.* The regimen of MGTA-145 and plerixafor was well tolerated. Treatment emergent pain was seen in 44% of patients (11/25). Acute, transient, MGTA-145-related grade 1 bone or musculoskeletal pain was observed in 38% of patients (9/25) shortly after MGTA-145 infusion, resolving within seven minutes for all patients.
- *Engraftment.* All transplanted patients (18/18), evaluable as of the cutoff date, successfully engrafted. Neutrophils recovered after a median of 12 days and platelets after a median of 17.5 days, which are comparable to historical data. Red blood cell transfusion was needed in 17% of patients (3/25).
- *100 Day Follow-Up.* All 14 transplanted patients as of the data cut-off date had completed day-100 follow up with durable engraftment.
- *CD34+CD90+ Cells.* The collected CD34+ stem cells contain a high percentage of CD34+CD90+ cells, a stem cell population associated with multi-lineage, long-term engraftment. 74% of grafts (17/23) were negative for minimal residual disease using next generation flow cytometry.

Next Steps in Multiple Myeloma

As described in the company's third quarter earnings release, the results from this investigator-initiated trial represent a positive step forward in the

development of MGTA-145, in combination with plerixafor, as a potential first line stem cell mobilization regimen. Based on the encouraging collection and engraftment data, the company intends to explore further development of MGTA-145 in a Phase 2b clinical setting. This approach would enable a comprehensive evaluation of the multiple myeloma patient population and allow for adjustments of dosing and administration which the company, in both cases, has identified as opportunities for optimization as a result of this investigator-initiated study and the company's other MGTA-145 development efforts.

"While Dr. Sidana and her team are collecting and analyzing additional patient-level data, these topline results are encouraging and support further development of MGTA-145." commented Dr. Jeffrey Humphrey, M.D., the company's Chief Medical Officer. "We believe this novel mobilization regimen has the potential to replace G-CSF regimens and to enable reliable, predictable, rapid and well-tolerated mobilization of stem cells for both transplant and gene therapies."

MGTA-145 is also being evaluated for its ability to mobilize stem cells for collection from donors for allogenic transplantation in patients with acute lymphoblastic leukemia (ALL), acute myeloid leukemia (AML) and myelodysplastic syndromes (MDS) in a Phase 2 clinical trial. The company is planning to open an additional Phase 2 clinical trial for mobilization and collection of stem cells for patients with sickle cell disease in December 2021.

Antibody-Drug Conjugate (ADC) Targeted Conditioning Program

Oral Presentation Showcasing Non-human Primate Data of Targeted ADC Conditioning for Gene Therapy

Title: CD117 Antibody Drug Conjugate-Based Conditioning Allows for Efficient Engraftment of Gene-Modified CD34+ Cells in a Rhesus Gene Therapy Model (Oral Abstract #560)

Presenting Author: Naoya Uchida, M.D., National Institutes of Health

Date: Sunday, December 12, 2021, 4:45pm ET

This preclinical study evaluated escalating doses of a tool CD117-ADC. As monotherapy conditioning, a single dose of the CD117-ADC allowed for efficient engraftment of gene-modified autologous stem cells in a rhesus model of gene therapy, without chemotherapy or radiation conditioning. Engraftment of gene-modified stem cells achieved with monotherapy CD117-ADC was robust and durable, equivalent to that achieved with four doses of myeloablative busulfan conditioning. Sustained gene expression of hemoglobin F was confirmed at the protein level in this CD117-ADC-conditioned rhesus transplant model of gene therapy for sickle cell disease. Compared to chemotherapy or radiation-based conditioning regimens, conditioning with monotherapy CD117-ADC could be both sufficiently potent and well tolerated to improve the safety and risk benefit profile for gene therapies that require stem cell transplantation.

Poster Presentation Highlighting Preclinical Data of Targeted ADC Conditioning Program:

Title: CD117-Targeted ADC, in Combination with Lymphodepleting Antibodies, Enables Allogeneic Hematopoietic Stem Cell Transplantation in Mice without Chemotherapy or Radiation (Poster #1682)

Presenting Author: Leanne Lanieri, M.S., Magenta Therapeutics, Inc.

Date to View Poster Presentation: Saturday, December 11, 2021, 5:30pm – 7:30pm ET

This study evaluated the combination of a tool CD117-ADC with lymphodepleting antibodies as the conditioning regimen in a murine model of allogeneic HSC transplantation. The targeted conditioning regimen enabled complete donor chimerism in a fully mismatched allogeneic HSC transplant murine model, without use of chemotherapy or radiation. Antibody-based targeted conditioning regimens could offer a more favorable risk-benefit profile over chemotherapy and radiation-based conditioning regimens. An improved risk benefit profile, in turn, could extend the curative potential of allogeneic HSC transplantation to more patients with malignant and non-malignant diseases who otherwise would not be eligible for HSC transplantation.

About Magenta Therapeutics

Magenta Therapeutics is a clinical-stage biotechnology company developing medicines designed to bring the curative power of stem cell transplants to more patients with blood cancers, genetic diseases and autoimmune diseases. Magenta is combining leadership in stem cell biology and biotherapeutics development with clinical and regulatory expertise and broad networks in the stem cell transplant community to revolutionize immune reset for more patients.

Magenta is based in Cambridge, Massachusetts. For more information, please visit www.magentatx.com.

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This press release may contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, 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 initiation of clinical trials or the results of ongoing and planned clinical trials, the development of product candidates and advancement of preclinical programs, projections regarding future revenues and financing performance, long-term growth, cash, cash equivalents and marketable securities, the anticipated timing of clinical trials and regulatory filings, the potential benefits of product candidates, the timing, progress and success of collaborations, as well as other statements containing the words "anticipate," "believe," "continue," "could," "endeavor," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "seek," "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 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; discussions with governmental agencies such as the FDA; 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 continuing COVID-19 pandemic on Magenta's business, operations, strategy, goals and anticipated timelines, Magenta's ongoing and planned preclinical activities, Magenta's ability to initiate, enroll, conduct or complete ongoing and planned clinical trials, Magenta's timelines for regulatory submissions and Magenta's financial position; 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, 2021, as updated by Magenta's 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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