Magenta Therapeutics Announces Commencement of First Phase 2 Clinical Trial of MGTA-145 for Stem Cell Mobilization, Oral Presentation of MGTA-145 Phase 1 Results and Update on Targeted ADC Conditioning Program MGTA-117 at ASH Annual Meeting

December 7, 2020

– Enrollment commenced in the MGTA-145 Phase 2 clinical trial of autologous transplant of multiple myeloma patients at Stanford University –

– Oral presentation of Phase 1 clinical data presented at the 62nd American Society of Hematology (ASH) Annual Meeting confirming MGTA-145 achieved proof-of concept: all safety and efficacy endpoints met and mobilized cells demonstrated functional superiority over other mobilization approaches in preclinical studies –

– Preclinical data from MGTA-117, the first targeted antibody-drug conjugate (ADC) from the Magenta platform, continue to indicate that it is an effective, potent conditioning agent with the potential to improve transplant outcomes in patients with blood cancers and genetic diseases –

– Magenta expects to provide additional updates on its programs and clinical plans in early 2021 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 7, 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 announced final clinical results from its earlier completed Phase 1 clinical trial as well as development updates for its MGTA-145 stem cell mobilization therapy, including commencement of enrollment in a Phase 2 clinical trial in multiple myeloma, and its plans for a Phase 2 clinical trial in allogeneic stem cell transplant for patients with acute myeloid leukemia (AML), acute lymphocytic leukemia (ALL) and myelodysplastic syndrome (MDS). The company also previously announced a clinical collaboration with bluebird bio to evaluate MGTA-145 for mobilizing and collecting stem cells in adults and adolescents with sickle cell disease (SCD). Additional preclinical results were also presented at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition, taking place virtually from December 5-8, 2020, on the Magenta conditioning platform, including MGTA-117 program, which is a targeted antibody-drug conjugate (ADC) to prepare patients for stem cell transplant.

MGTA-145 Advancement to Phase 2 Development in Blood Cancers

The company announced that enrollment has started and is ongoing in a Phase 2 clinical trial of MGTA-145, used in combination with plerixafor, to mobilize and collect stem cells for autologous stem cell transplantation of multiple myeloma patients at Stanford University. Magenta expects that this trial will provide patient-level data on stem cell mobilization and collection, characteristics of the mobilized graft and engraftment in patients with multiple myeloma.

Additionally, through a collaboration with the National Marrow Donor Program®/Be The Match®, a global leader in facilitating allogeneic hematopoietic stem cell transplantation, Magenta plans to initiate a Phase 2 clinical trial in early 2021 using MGTA-145 to mobilize and collect stem cells from allogeneic donors for transplant in patients with AML, ALL and MDS. Allogeneic stem cell transplant provides a potentially curative therapeutic option for patients with these diseases. This clinical trial will evaluate stem cell mobilization, collection, cell quality, engraftment and the potential for reduced Graft-versus-Host Disease (GvHD), which is of particular importance in the allogeneic transplant setting.

MGTA-145 in Sickle Cell Disease

Magenta Therapeutics recently announced an exclusive clinical collaboration with bluebird bio to evaluate the utility of MGTA-145, in combination with plerixafor, for the mobilization and collection of stem cells in adults and adolescents with SCD.

The data from this clinical trial could provide proof-of-concept for MGTA-145, in combination with plerixafor, as the preferred mobilization regimen for patients with SCD. bluebird bio's experience with plerixafor as a mobilization agent in SCD aligns with Magenta's combination therapy approach, utilizing MGTA-145 plus plerixafor with potential for safe, rapid and reliable mobilization of sufficient quantities of high-quality stem cells to improve outcomes associated with stem cell transplantation.

MGTA-145 Presentations at ASH

Magenta presented final clinical data from its MGTA-145 stem cell mobilization Phase 1 clinical trial in healthy volunteers at the ASH Annual Meeting. All primary and secondary endpoints were met in the study completed earlier this year.

The results demonstrate that a single dose of MGTA-145, in combination with plerixafor, rapidly and reliably mobilized high numbers of stem cells in a single day without the need for G-CSF for potential use in diseases that can benefit from autologous and/or allogeneic stem cell transplantation. The additional data also offer further confirmation that MGTA-145, in combination with plerixafor, was well tolerated and provides a rapid and reliable method to obtain large numbers of hematopoietic stem cells. Transplant of these cells in preclinical models resulted in enhanced, durable engraftment, in addition to highly immunosuppressive properties, leading to reduced GvHD.

"Results from this study provide a robust dataset and proof of concept that MGTA-145, in combination with plerixafor, provides rapid and robust mobilization of stem cells and that these cells have better engraftment potential, are able to be gene modified and engraft and reduce GvHD in preclinical models compared to cells mobilized with other available agents. The data reinforce the availability of compelling opportunities for development in both the autologous and allogeneic transplant settings," said John Davis Jr., M.D., M.P.H., M.S., Head of Research & Development and Chief Medical Officer, Magenta Therapeutics.

The data were presented by Steven M. Devine, MD, Chief Medical Officer of the National Marrow Donor Program/Be The Match and Associate Scientific Director of the CIBMTR® (Center for International Blood and Marrow Transplant Research®).

Conditioning Program (MGTA-117 and CD45-ADC) Presentations at ASH

Magenta also provided updates on its conditioning platform at the ASH Annual Meeting, including MGTA-117 and CD45-ADC programs. Preclinical data from a study of MGTA-117 demonstrate that it is an effective, potent conditioning agent for transplant with anti-leukemic activity, significantly decreasing tumor burdens, leading to delayed tumor growth and increased median survival rates in animal models of AML. Ongoing GLP toxicology and GMP manufacturing progress continue to be supportive of advancing MGTA-117 towards an IND filing in AML and MDS.

Additionally, preclinical data from a study of Magenta's CD45-ADC, a CD45-targeted conditioning agent designed to remove the cells that cause autoimmune diseases to enable curative immune reset, demonstrated the ability to achieve successful outcomes as a single agent in the most challenging disease model through fully mismatched allogeneic hematopoietic stem cell transplant, where only radiation or combinations of toxic chemotherapies are available, potentially providing patients the option of a reduced toxicity conditioning regimen. The company continues to evaluate this program preclinically.

About MGTA-145

MGTA-145 is being developed in combination with plerixafor to harness complementary chemokine mechanisms to mobilize hematopoietic stem cells for collection and transplantation. This new combination has the potential to be the preferred mobilization regimen for rapid and reliable mobilization and collection of hematopoietic stem cells to improve outcomes in autologous and allogeneic stem cell transplantation, which can rebuild a healthy immune system for patients with blood cancers, genetic diseases and autoimmune disorders.

MGTA-145 has the potential to replace the current standard of care for patients and allogeneic donors who currently rely on the use of granulocyte-colony stimulating factor (G-CSF) alone or in combination with plerixafor, which can take up to five days or longer to mobilize sufficient numbers of stem cells, often resulting in significant bone pain and other side effects.

About Magenta Therapeutics

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

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

Follow Magenta on Twitter: @magentatx.

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 filed on March 3, 2020, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20201207005269/en/

Magenta Therapeutics:
Lyndsey Scull, Director, Corporate Communications, Magenta Therapeutics
202-213-7086
lscull@magentatx.com

Investor inquiries:
Jill Bertotti, W2O Group
714-225-6726
jbertotti@w2ogroup.com

Media inquiries:
Dan Budwick
1AB
dan@1abmedia.com

Source: Magenta Therapeutics