Oral Presentations Showcasing Clinical Data of MGTA-145 Stem Cell Mobilization Program

Magenta is developing MGTA-145 in combination with plerixafor utilizing complementary mechanisms to mobilize hematopoietic stem cells (HSCs) for collection and transplantation. This combination has the potential to be the preferred mobilization regimen for rapid, reliable, predictable and safe collection of high numbers of functional blood stem cells to improve outcomes across autologous and allogeneic stem cell transplantation, which also includes stem cells necessary for all HSC-based gene therapies.

**Title:** MGTA-145 / Plerixafor-Mediated HSC Mobilization and Intravenous HDAd5/35++ Vector Injection into Mice Allows for Efficient in vivo HSC Transduction and Stable Gene Marking in Peripheral Blood Cells (Oral Abstract, #16)

**Presenting Author:** Chang Li, Ph.D., Division of Medical Genetics, Department of Medicine, University of Washington

**Date and Time of Presentation:** Session B – Transplantation for Non-Malignant Disease; Monday, February 8, 2021, 3:15PM CST / 4:15PM EST

Data from this preclinical study demonstrate the potential of MGTA-145 plus plerixafor to serve as an efficient, single-dose mobilization regimen for in vivo HSC gene therapy where stem cells could be gene corrected or edited without having to remove them from the body. This could potentially replace current mobilization regimens that rely on ex vivo gene therapy approaches to treat genetic diseases.

**Title:** MGTA-145, in Combination with Plerixafor in a Phase 1 Clinical Study, Mobilizes Large Numbers of Hematopoietic Stem Cells and a Graft with Potent Immunosuppressive Properties for Autologous and Allogeneic Transplant (Oral Abstract, #35)

**Presenting Author:** Kevin Goncalves, Ph.D., Magenta Therapeutics

**Date and Time of Presentation:** Session E – Consider the Source: Stem Cell Grafts and Donors; Tuesday, February 9, 2021, 4:00PM CST / 5:00PM EST

Data from this Phase 1 clinical trial with healthy volunteers further underscore the potential utility of MGTA-145 plus plerixafor as an effective, single-day mobilization and collection regimen for autologous and allogeneic HSC transplant. MGTA-145 plus plerixafor mobilized high numbers of HSCs and showed durable engraftment, successful gene-modification and immunosuppressive properties by reducing Graft-versus-Host disease (GvHD) in preclinical models.

Oral Presentation Showcasing Preclinical Study of MGTA-117 Targeted ADC Conditioning Program

Magenta is developing a suite of novel antibody-drug conjugates (ADCs) for conditioning, a step in the transplant process that currently relies on the use of systemic chemotherapy agents and radiation. Magenta’s targeted conditioning programs are designed to selectively eliminate stem cells and/or immune cells from a patient prior to transplant or gene therapy, and to reduce or potentially eliminate the need for high dose or high intensity chemotherapy-based treatments. These programs focus on developing targeted products that remove specific cell types, with an approach that is tailored to the patient’s disease and transplant requirements.

MGTA-117, Magenta’s most advanced conditioning program, is a CD117-targeted ADC designed to precisely deplete hematopoietic stem and progenitor cells to clear space in the bone marrow prior to transplant, and to support long-term engraftment and improved disease outcomes in patients. MGTA-117 has shown to be highly selective for potent activity, efficacy and tolerability in preclinical models.

**Title:** A Single Dose of a Novel Anti-Human CD117-Amanitin Antibody Drug Conjugate (ADC) Engineered for a Short Half-life Provides Dual Conditioning and Anti-Leukemia Activity and Extends Survival Compared to Standard of Care in Multiple Pre-clinical Models of Acute Myeloid Leukemia (AML) (Oral Abstract, #53)

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

**Date and Time of Presentation:** Session H – Novel Conditioning Regimens & Transplantation for Aged Populations, Wednesday, February 10, 2021, 4:00PM CST / 5:00PM EST

Hematopoietic stem cell transplant (HSCT) can often be a curative treatment for patients with acute myeloid leukemia (AML). There is currently a need for safer and more effective targeted conditioning agents, as current conditioning regimens are associated with severe toxicities and high post-transplant relapse or graft failure. MGTA-117 was studied in multiple human leukemic xenograft murine models to mimic untreated and refractory AML. In preclinical models, MGTA-117 significantly increased median survival versus a multi-day standard-of-care regimen using cytarabine. Data from this study demonstrate MGTA-117’s potential as a potent, targeted HSCT conditioning agent with anti-leukemic activity, emphasizing its potential
Targeted CD45 Antibody Drug Conjugate Enables Full Mismatch Allogeneic Hematopoietic Stem Cell Transplantation in a Murine HSCT Model

Magenta’s other ADC-based conditioning program, CD45-ADC, targets both patient HSCs and disease-causing immune cells. The program’s lead target is CD45, a cell surface molecule broadly expressed throughout the hematopoietic and immune systems. CD45-ADC has the potential to significantly increase the number of patients eligible to receive a stem cell transplant, particularly those patients with autoimmune diseases and acute leukemias.

Developing a broad targeting approach for safer patient conditioning prior to HSCT could bring the curative potential of allogeneic HSCT to more patients with both malignant and non-malignant disorders. Current conditioning regimens limit accessibility of this procedure due to toxicity.

Title: Targeted CD45 Antibody Drug Conjugate Enables Full Mismatch Allogeneic Hematopoietic Stem Cell Transplantation in a Murine HSCT Model as a Single Agent (AML) (Poster #242)

Lead Author: Sharon Hyzy, M.S., Magenta Therapeutics

Data from this study showed conditioning with single agent CD45-ADC enabled complete chimerism in a full mismatch allogeneic HSCT model.

Oral Presentation of MGTA-456 Stem Cell Therapy Expansion Program in Patients with Blood Cancer

Magenta is continuing long-term patient follow up to evaluate MGTA-456 in blood cancers through the investigator-initiated Phase 2 trial in blood cancers at the University of Minnesota and will assess best next steps for the program. Magenta previously announced in June 2020 it had discontinued enrollment in the Phase 2 trial of MGTA-456 in patients with inherited metabolic disorders.

Title: MGTA-456, A CD34 Expanded Cord Blood Product, Permits Selection of Better HLA Matched Units and Results in Rapid Hematopoietic Recovery, Uniform Engraftment and Reduced Graft-Versus-Host Disease in Adults with High-Risk Hematologic Malignancies (Oral Abstract, #31)

Presenting Author: Heather Stefanski, M.D., Ph.D., Assistant Professor, Department of Pediatrics, University of Minnesota

Date and Time of Oral Presentation: Session E – Consider the Source: Stem Cell Grafts and Donors; Tuesday, February 9, 2021, 3:00PM CST / 4:00PM EST

Twenty-two patients were enrolled in the study, with 18 transplanted with MGTA-456. Compared to transplant patients who had undergone the same conditioning, GvHD prophylaxis and supportive care, patients who received MGTA-456 showed faster neutrophil recovery (median of 17 days compared to 23 days) and better platelet recovery (median 36 days compared to 59 days). Additionally, incidence of grade 2-4 acute GvHD was lower (24% compared to 46%), likely because of the ability to find a better matched cord unit.

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 autoimmunity 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 community to revolutionize immune reset for more patients.

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

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