Magenta Therapeutics Announces Acceptance of Nine Abstracts Covering Clinical and Preclinical Data at ASH, Representing Progress across the Portfolio of Conditioning, Stem Cell Mobilization and Expansion Programs

November 1, 2018

- Non-genotoxic antibody drug conjugates (ADCs) potently depleted human stem cells in five abstracts on targeted conditioning programs and demonstrated survival benefit in patient-derived leukemia models
- MGTA-145 robustly mobilized cells that block graft-versus-host disease in preclinical models in addition to large numbers of hematopoietic stem cells that engrafted in primates following apheresis
- All five patients with inherited metabolic disorders (IMDs) treated with MGTA-456 in ongoing Phase 2 study met the primary endpoint of successful engraftment by day 42, with median of one day of neutropenia
- Company intends to launch a Phase 2 study of MGTA-456 in patients with severe sickle cell disease in the first half of 2019, and investigator-initiated Phase 2 study in blood cancers on track to begin in late 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 1, 2018--Magenta Therapeutics (NASDAQ: MGTA), a clinical-stage biotechnology company developing novel medicines to bring the curative power of bone marrow transplant to more patients, today announced that the Company will present clinical data and preclinical research at the 60th annual meeting of the American Society of Hematology (ASH), taking place December 1st through 4th in San Diego, Calif. Preliminary data in these abstracts became available on the ASH conference website at 9:00 a.m. ET today. The Company also provided an update today on its ongoing Phase 2 study of MGTA-456 in inherited metabolic disorders, including recent data from the study.

ASH Abstracts

“Magenta is developing potentially transformative drugs to broaden the reach of one-time, curative cell therapies – including bone marrow transplant, haploidentical transplant, cell and gene therapy and genome editing – to more patients with devastating diseases, including blood cancers, genetic diseases and autoimmune diseases,” said Jason Gardner, D.Phil., president, chief executive officer and co-founder, Magenta Therapeutics. “This year’s nine data presentations at ASH highlight our patient conditioning, mobilization and stem cell expansion programs, giving further insight into our progress in addressing the major unmet needs of the transplant process, including toxic conditioning regimens, inadequate stem cell mobilization and low cell doses. We anticipate building on this progress over the coming year as we move our mobilization program into the clinic and advance our clinical development plan for stem cell expansion in multiple diseases.”

Conditioning Programs

CD117-Amanitin Antibody Drug Conjugates Effectively Deplete Human and Non-Human Primate HSCs: Proof of Concept as a Targeted Strategy for Conditioning Patients for Bone Marrow Transplant, Abstract #3314

Presenter: Brad Pearse, Ph.D., Magenta Therapeutics
Session Name: 701. Experimental Transplantation: Basic Biology, Pre-Clinical Models: Poster II
Session Date: Sunday, December 2, 2018
Session Time: 6:00 PM - 8:00 PM
Room: San Diego Convention Center, Hall GH

Targeting CD45 with an Amanitin Antibody-Drug Conjugate Effectively Depletes Human HSCs and Immune Cells for Transplant Conditioning, Abstract #4526

Presenter: Rahul Palchaudhuri, Ph.D., Magenta Therapeutics
Session Name: 701. Experimental Transplantation: Basic Biology, Pre-Clinical Models: Poster III
Session Date: Monday, December 3, 2018
Session Time: 6:00 PM - 8:00 PM
Room: San Diego Convention Center, Hall GH

Single Doses of Antibody Drug Conjugates (ADCs) Targeted to CD117 or CD45 Have Potent In Vivo Anti-Leukemia Activity and Survival Benefit in Patient Derived AML Models, Abstract #3316

Presenter: Jennifer Proctor, Magenta Therapeutics
Session Name: 701. Experimental Transplantation: Basic Biology, Pre-Clinical Models: Poster II
Session Date: Sunday, December 2, 2018
Session Time: 6:00 PM - 8:00 PM
Room: San Diego Convention Center, Hall GH

Antibody Drug Conjugates Targeted to CD45 or CD117 Enable Allogeneic Hematopoietic Stem Cell Transplantation in Animal Models, Abstract #3324

Presenter: Rahul Palchaudhuri, Ph.D., Magenta Therapeutics
Session Name: 701. Experimental Transplantation: Basic Biology, Pre-Clinical Models: Poster II
Date: Sunday, December 2, 2018
Presentation Time: 6:00 PM - 8:00 PM
Location: San Diego Convention Center, Hall GH
Non-genotoxic conditioning facilitates robust HSPC engraftment and multi-lineage development in a dose dependent manner in Fanconi anemia

Presenter: Meera Srikanthan, Ph.D., Fred Hutchinson Cancer Research Center
Session Name: TBA
Date: TBA
Presentation Time: TBA
Location: TBA

Preclinical Data, Stem Cell Mobilization Program

MGTA-145 In Combination with Plerixafor Rapidly Mobilizes High Numbers of Hematopoietic Stem Cells and Graft-Versus-Host Disease Inhibiting Myeloid-Derived Suppressor Cells in Non-Human Primates, Abstract #116

Presenter: Kevin Goncalves, Ph.D., Magenta Therapeutics
Session Name: 711. Cell Collection and Processing I
Session Date: Saturday, December 1, 2018
Session Time: 9:30 AM - 11:00 AM
Presentation Time: 9:45 AM
Room: Manchester Grand Hyatt San Diego, Grand Hall A

Clinical Data, MGTA-456 Stem Cell Expansion Program

Preliminary Phase 2 Results Demonstrate Engraftment with Minimal Neutropenia with MGTA-456, a CD34+ Expanded Cord Blood Product in Patients Transplanted for Inherited Metabolic Disorders, Abstract #3467

Presenter: John Wagner, M.D., Director, Blood and Marrow Transplantation Division, University of Minnesota
Session Name: 732. Clinical Allogeneic Transplantation: Results: Poster II
Session Date: Saturday, December 1, 2018
Session Time: 6:00-8:00 p.m. PT
Room: San Diego Convention Center, Hall GH

MGTA-456 Contains Large Numbers of Expanded Cord Blood (CB) CD34+CD90+ Hematopoietic Stem Cells (HSC) Which Confer All Engraftment Activity and Correlate with Accelerated Neutrophil Recovery after Myeloablative Conditioning in Patients with Hematologic Malignancy, Abstract #2083

Presenter: Tony Boitano, Ph.D., Magenta Therapeutics
Session Name: 721 Clinical Allogeneic Transplantation: Conditioning Regimens, Engraftment, and Acute Transplant Toxicities
Session Date: Saturday, December 1, 2018
Session Time: 6:15-8:15 p.m. PT
Room: San Diego Convention Center, Hall GH

Preclinical Data, MGTA-456 Stem Cell Expansion Program

MGTA-456, A First-in-Class Cell Therapy Produced from a Single Cord Blood Unit, Enables A Reduced Intensity Conditioning Regimen and Enhances Speed and Level of Human Microglia Engraftment in the Brains of NSG Mice, Abstract #115

Presenter: Kevin Goncalves, Ph.D., Magenta Therapeutics
Session Name: 711. Cell Collection and Processing
Session Date: Saturday, December 1, 2018
Presentation Time: 9:30 a.m. PT
Room: San Diego Convention Center, Grand Hall A

MGTA-456 Clinical Development Update

MGTA-456 is a cell therapy providing a high dose of hematopoietic stem cells that are well-matched to the patient, administered through a transplant procedure. The ongoing Phase 2 study in inherited metabolic disorders aims to enroll 12 patients with Hurler’s syndrome, cerebral adrenoleukodystrophy (cALD), metachromatic leukodystrophy or globoid cell leukodystrophy. The primary endpoint is engraftment after transplantation and the secondary endpoint is transplant-related safety and tolerability.

The data as of October 22, 2018 show:

- Five patients treated and evaluable: three with Hurler’s syndrome, two with cALD
- Five of five patients treated with MGTA-456 met the primary endpoint of successful engraftment by day 42 following the transplant procedure.
  - In recent historical cohorts of patients undergoing regular cord blood transplantation with identical pre-transplant conditioning, up to 32% did not engraft at comparable time points.
- The patients treated with MGTA-456 had minimal neutropenia, lasting for a median of 1 day.
  - In the historical cohort, neutropenia lasted for a median of 8 days.
- Two patients less than 2 years of age with Hurler’s syndrome treated with MGTA-456 in the Magenta study developed autoimmune cytopenia, a known and frequent complication of transplant in these younger patients and patients with Hurler’s syndrome.
  - The first patient subsequently died from this complication. This was determined to be not related to the MGTA-456 product.
The second patient is currently undergoing treatment for the autoimmune cytopenia.

“Blood stem cell transplantation remains the standard of care for inherited metabolic disorders, which if untreated are generally progressive and lethal. Unfortunately, transplantation remains a difficult and complex procedure,” said Paul Orchard, M.D., Medical Director of the Inherited Metabolic & Storage Disease Bone Marrow Transplantation Program, University of Minnesota and principal investigator in the MGTA-456 study. “Many patients experience significant and life-threatening complications, such as dysregulation of the immune system following transplantation, including the emergence of antibodies to components of the blood system. Patients with inherited metabolic disorders, particularly patients with Hurler’s syndrome, are at higher risk for these antibody-associated cytopenias. In a prior transplant protocol using the identical combination of chemotherapy agents for pre-transplant conditioning, evidence of cytopenias was present in 9 of 15 patients under 2 years of age (53%). This has proven less common in older patients.”

In light of the elevated incidence of autoimmune cytopenias in very young patients and patients with Hurler’s syndrome, the Company announced today that it has voluntarily focused future enrollment towards pediatric patients older than 2 years of age diagnosed with leukodystrophies. The Company plans to continue enrolling patients with leukodystrophies in the study.

‘MGTA-456 is a first-in-class cell therapy with high doses of stem cells, and larger doses of stem cells are correlated with more successful transplant outcomes and faster time to engraftment and immune recovery. It has demonstrated engraftment and robust immune recovery in all 41 evaluable patients that have been treated to date in our Phase 2 study in inherited metabolic disorders and our Phase 1/2 study in blood cancers,” said John Davis, M.D., M.P.H., chief medical officer, Magenta Therapeutics. “We are developing MGTA-456 for patients with a broad range of diseases, including leukodystrophies, sickle cell disease and blood cancers, where we believe it has the potential to deliver transformative benefit.”

The Company is on track to initiate a Phase 2 study of MGTA-456 in patients with sickle cell disease in the first half of 2019, and an investigator-initiated Phase 2 study in blood cancers is on track to start in 2018.

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, including express or implied statements regarding Magenta’s future expectations, plans and prospects, including projections regarding future revenues and financing performance, our long-term growth, the anticipated timing of our clinical trials and regulatory filings, the development of our product candidates and advancement of our preclinical programs, as well as other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “project,” “should,” “target,” “will” or “would” and similar expressions that constitute forward-looking statements under the Private Securities Litigation Reform Act of 1995. 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. These and other risks concerning Magenta’s programs and operations are described in additional detail in its registration statement on Form S-1, its Quarterly Report on Form 10-Q and its other filings made with the Securities and Exchange Commission from time to time. 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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