Magenta Therapeutics to Present Data from Across Portfolio at American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

April 28, 2020

--Magenta will present seven abstracts showcasing findings across clinical and pre-clinical pipeline, including five oral and two poster presentations--

--MGTA-145 clinical data will demonstrate rapid, single-day mobilization of large numbers of human HSCs that can be gene-modified and result in superior engraftment in pre-clinical models compared to standard-of-care--

--CD117-ADC data will demonstrate successful gene therapy transplant and long-term HSC engraftment in non-human primates via single dose of targeted ADC without chemotherapy conditioning--

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 28, 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 that data from across its portfolio will be presented at the ASGCT annual meeting, to be held May 12-15, 2020.

“Magenta’s pipeline continues to deliver new results and continues our progress toward our goal of allowing all patients who can benefit to receive curative stem cell gene therapy or transplant. Our ASGCT presentations demonstrate the far-reaching potential of our programs to widen availability of and improve the patient experience with stem cell gene therapy and transplant,” said John Davis Jr., M.D., M.P.H., M.S., Head of Research & Development and Chief Medical Officer, Magenta Therapeutics. “We are particularly encouraged by the new results on our first-line mobilization and conditioning medicines for patients.”

Data from MGTA-145 First-Line Stem Cell Mobilization Program:

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, blood cancers and genetic diseases, such as sickle cell disease. MGTA-145, a CXCR2 agonist, works in combination with plerixafor, a CXCR4 antagonist, to rapidly and robustly mobilize stem cells for collection and transplant. These data provide further confirmation that MGTA-145, in combination with plerixafor, enables the same-day mobilization of functional hematopoietic stem cells (HSCs) that can be gene-modified and engrafted.

Title: MGTA-145 in Combination with Plerixafor, Rapidly Mobilizes Large Numbers of HSCs in Humans That Can Be Gene Edited with CRISPR/Cas9 and Mediate Superior Engraftment to Standard-of-Care (Abstract #123)

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

Date and Time: Tuesday, May 12, 2020 – 3:45-5:30pm

This abstract demonstrates that MGTA-145 plus plerixafor is a rapid, reliable, efficient medicine to obtain high numbers of HSCs that can be gene edited with CRISPR/Cas9 and mediate durable engraftment in preclinical models.

Title: MGTA-145/Plerixafor-Mediated HSC Mobilization and Intravenous Gene Therapy in Mice Allows for Efficient in vivo HSC Transduction and Stable Gene Marking in Peripheral Blood Cells (Abstract #810)

Presenter: Chang Li, Ph.D., Division of Medical Genetics, Department of Medicine, University of Washington

Date and Time: Wednesday, May 13, 2020 – 5:30-6:30pm

This abstract shows, for the first time, that MGTA-145 plus plerixafor can enable robust mobilization of large numbers of stem cells in animals that can be efficiently modified in vivo by gene therapy without transplant.

Data from CD117-ADC Gene Therapy Conditioning Program

Targeted, disease-modifying antibody drug conjugates (ADCs) are designed to precisely and rapidly remove disease-causing cells in the body and enable immune and blood system reset and long-term engraftment, without the need for chemotherapy or radiation. The results to be presented at ASGCT use a tool CD117-ADC molecule to demonstrate the first-ever successful transplant of gene-modified cells in non-human primates without the use of chemotherapy or radiation. Magenta has built on these results to declare its clinical candidate, MGTA-117, for targeted patient preparation for stem cell gene therapy and transplant. Magenta is on track to deliver initial clinical data on MGTA-117 in 2021.

Title: A Single Dose of Fast Half-Life CD117 Antibody Drug Conjugate Enables Hematopoietic Stem Cell-Based Gene Therapy in Nonhuman Primates (Abstract #533)

Presenter: Naoya Uchida, M.D., Ph.D., Cellular and Molecular Therapeutics Branch; National Heart, Lung, and Blood Institute; National Institutes of Health

Date and Time: Wednesday, May 13, 2020 – 3:45-5:30pm

This abstract demonstrates that a single dose of a tool CD117-ADC selectively depleted HSCs in non-human primates while sparing immune cells, which are important for recovery following transplant. A single dose of CD117-ADC in non-human primates enabled successful transplant and long-term engraftment of HSCs modified with a lentiviral vector encoding the β-globin gene, the gene that causes sickle cell disease and β-thalassemia, with none of the side effects associated with busulfan conditioning.

Additional Posters and Presentations

Title: Expansion with E478 Significantly Increases the Rate of CRISPR-Mediated Homology Directed Repair (HDR) and Improves Engraftment of
Human Hematopoietic Stem Cells (Abstract #10)
**Presenter:** Kevin Goncalves, Ph.D., Magenta Therapeutics, Cambridge, Mass.
**Date and Time:** Tuesday, May 12, 2020 – 10:15am-12:00pm

**Title:** MGTA-456, A Cell Therapy Utilizing an Aryl Hydrocarbon Receptor Antagonist (AHRa) Culture, Promotes Expansion of CD34+CD90+Cord Blood (CB) Hematopoietic Stem Cells (HSC), Resulting in Rapid Hematopoietic Recovery, Uniform Engraftment and Better HLA Matched Grafts for Larger Recipients (Abstract #120)
**Presenter:** John Wagner, M.D., Executive Medical Director, Bone Marrow Transplant Program, University of Minnesota, Minneapolis, Minn.
**Date and Time:** Tuesday, May 12, 2020 – 3:45-5:30pm

**Title:** High Dose Hematopoietic Stem Cell Therapies, like MGTA-456, Enable Complete Neural, Peripheral and Skeletal Disease Cross-Correction Through Rapid and Robust Engraftment (Abstract #248)
**Presenter:** Sharon Hyzy, M.S., Magenta Therapeutics, Cambridge, Mass.
**Date and Time:** Tuesday, May 12, 2020 – 5:30-6:30pm

**Title:** A Phase 2 Trial of MGTA-456 Cell Therapy Demonstrates Rapid and Durable Long-Term Improvement in Disease-Specific Outcomes in Inherited Metabolic Disease (IMD) Patients (Abstract #1302)
** Presenter:** John Wagner, M.D., Executive Medical Director, Bone Marrow Transplant Program, University of Minnesota, Minneapolis, Minn.
**Date and Time:** Friday, May 15, 2020 – 8:00-9:45am

### 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 autoimmune diseases, genetic diseases and blood cancers. 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](http://www.magentatx.com).

Follow Magenta on Twitter: [@magentatx](https://twitter.com/magentatx).

### 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, without limitation, statements regarding expectations and plans for presenting pre-clinical and clinical data, projections regarding future revenues and financing performance, our long-term growth, cash, cash equivalents and marketable securities, 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. 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, including review under accelerated approval processes; orphan drug designation eligibility; 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; and other risks concerning Magenta's programs and operations are described in additional detail in its registration statement on Form S-1, its Annual Report on Form 10-K filed on March 19, 2019, its Quarterly Reports 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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Source: Magenta Therapeutics