



## **Magenta Therapeutics Highlights Recent Progress and Expected Timing of 2021 Milestones, Including Four Ongoing and Planned Clinical Trials**

January 11, 2021

-- MGTA-145: Three Phase 2 clinical trials ongoing or planned to evaluate MGTA-145, a biologic used in combination with plerixafor to mobilize stem cells; the first clinical trial in patients with multiple myeloma (initial data expected in mid-2021); the first clinical trial with matched donors and patients with acute myeloid leukemia (AML), acute lymphocytic lymphoma (ALL) and myelodysplastic syndromes (MDS) (data expected in the second half of 2021); and the first clinical trial in patients with sickle cell disease (trial initiation expected in the second half of 2021) --

-- MGTA-117: Completing GLP toxicology and GMP manufacturing of targeted conditioning antibody-drug conjugate, MGTA-117; plans to initiate clinical trial in acute myeloid leukemia and myelodysplastic syndromes in mid-2021 --

-- Five abstracts from across Magenta's pipeline, including four oral presentations, will be presented at the Transplantation and Cellular Therapy (TCT) Annual Meeting, to be held virtually February 8-12, 2021 --

-- Magenta also has announced the appointment of experienced biotech executive Alison Lawton to its Board of Directors --

-- Ended 2020 with cash reserves of approximately \$145 million that are expected to fund the current operating plan into 2023 --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 11, 2021-- **Magenta Therapeutics** (NASDAQ: MGTA), a clinical-stage biotechnology company developing novel medicines to bring the curative power of immune and blood systems reset via stem cell transplant to more patients, today highlighted progress across its stem cell mobilization and collection and targeted conditioning programs, and set expectations for 2021. These updates will be discussed during a webcast presentation at the 39th Annual J.P. Morgan Healthcare Conference on Thursday, January 14 at 7:50 a.m. PST / 10:50 a.m. EST.

"I'm exceptionally proud of the entire Magenta team who continued to adapt and execute across our portfolio, despite the disruptions that characterized 2020. This past year, we continued to drive our vision to bring immune and blood systems reset to more patients. We announced four pipeline-expanding partnerships, presented clinical and pre-clinical data across our pipeline and secured the capital that we expect can fund our operations into 2023. We continue to advance four ongoing and planned clinical trials that we believe can advance our portfolio in 2021 and, for MGTA-145 specifically, can provide proof-of-concept for stem cell mobilization across multiple diseases and the first clinical data for MGTA-117 targeted conditioning," said Jason Gardner, D. Phil., President and Chief Executive Officer, Magenta. "I am also delighted to welcome Alison Lawton's return to Magenta's Board of Directors. Alison brings extensive experience and leadership in both regulatory and business arenas, essential as the Magenta portfolio advances. We look forward to building on the momentum generated in 2020 as we relentlessly focus on execution."

### **Stem Cell Mobilization and Collection**

#### ***MGTA-145: Three Phase 2 Clinical Trials Ongoing or Planned***

**Autologous Stem Cell Transplant of Multiple Myeloma Patients.** Previously announced ongoing enrollment continues for the Phase 2 investigator-initiated clinical trial of MGTA-145, used in combination with plerixafor, to mobilize and collect stem cells for autologous stem cell transplantation in multiple myeloma patients at Stanford University. Magenta expects that this trial will provide data on stem cell mobilization and collection, durability of engraftment in transplanted patients and disease outcomes, including progression-free survival. Initial data from the study are expected in mid-2021.

**Allogeneic Donor Stem Cell Mobilization and Collection for Stem Cell Transplant in AML, ALL and MDS Patients.** Through a collaboration with the National Marrow Donor Program®/Be The Match®, Magenta plans to initiate, within the next several weeks, a Phase 2 clinical trial using MGTA-145 to mobilize and collect stem cells from allogeneic donors for transplant in patients with AML, ALL and MDS. This clinical trial will evaluate stem cell mobilization, collection, cell quality, engraftment and disease outcomes, including Graft-versus-Host Disease (GvHD), which is of particular importance in the allogeneic transplant setting. Initial data from this clinical trial are expected in the second half of 2021.

**Sickle Cell Disease – Stem Cell Mobilization and Collection; Cell Characterization; Pre-Clinical Gene Modification Model.** In collaboration with bluebird bio, Magenta plans to initiate a Phase 2 clinical trial in the second half of 2021 to evaluate MGTA-145, in combination with plerixafor, for the mobilization and collection of stem cells in adults and adolescents with sickle cell disease (SCD). Each party will characterize the cells and Magenta plans to gene-correct the cells and transplant them into established pre-clinical disease models to evaluate engraftment. 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 and, more broadly, across all gene therapy applications where safe, reliable and rapid mobilization of quality stem cells for gene-modification and transplant are necessary components.

#### ***About MGTA-145***

Magenta is developing MGTA-145 in combination with plerixafor to harness complementary mechanisms to mobilize hematopoietic stem cells (HSCs) for collection and transplantation. This combination has the potential to be the preferred mobilization regimen for safe, rapid and reliable mobilization and collection of HSCs and could improve outcomes in autologous and allogeneic stem cell transplantation.

### **Targeted Conditioning**

***MGTA-117: Plans to Initiate Phase 1 Clinical Trial in mid-2021; Initial Safety and Pharmacokinetics (PK) data to be assessed in the fourth quarter of 2021***

**AML and MDS.** Magenta is completing its IND-enabling GLP toxicology studies and GMP manufacturing process for MGTA-117, the first

antibody-drug conjugate (ADC) candidate from the company's research platform for targeted conditioning of patients prior to receiving a stem cell transplant for blood cancers or gene therapy drug products. Later this month, Magenta expects to complete its initial discussions with the U.S. Food and Drug Administration regarding the design of the first-in-human clinical trial. Magenta expects to file an Investigational New Drug (IND) application and, upon approval, plans to initiate a Phase 1 clinical trial in mid-2021 to assess the safety and PK in the first cohort of patients in the fourth quarter of 2021.

#### **About MGTA-117**

MGTA-117, Magenta's most advanced conditioning program, is a CD117-targeted antibody engineered for the transplant setting and conjugated to amanitin, a payload in-licensed from Heidelberg Pharma. MGTA-117 is designed to precisely deplete only hematopoietic stem and progenitor cells to clear space in the bone marrow prior to transplant, which supports long-term engraftment and disease outcomes in patients. MGTA-117 has shown high selectivity, potent efficacy, wide safety margins and broad tolerability in non-human primate models.

#### **Cash Guidance**

With focused allocation of resources on the Company's clinical trials and advancement of its research platform, the Company now believes its cash position will fund its operations into the first quarter of 2023.

#### **Alison Lawton Background**

Ms. Lawton is an executive leader with more than 30 years of experience in biopharma. She served as President and Chief Executive Officer of Kaleido Biosciences, Inc. (Nasdaq: KLDO) from August 2018 to June 2020, and served as President and Chief Operating Officer from December 2017 to August 2018. Prior to joining Kaleido Biosciences, Inc., Ms. Lawton served as Chief Operating Officer at Aura Biosciences, Inc., an oncology therapeutics company, from January 2015 until December 2017, and, prior to joining Aura, served as a consultant to Aura from March 2014 to December 2014. From January 2013 to January 2014, Ms. Lawton served as Chief Operating Officer at OvaScience Inc., a life sciences company. From 2014 to 2017, Ms. Lawton served as a biotech consultant for various companies, including as Chief Operating Officer consultant at X4 Pharmaceuticals. Prior to that, Ms. Lawton spent more than 20 years in various positions of increasing responsibility including Senior VP and General Manager of Biosurgery and prior, Senior VP of Market Access at Genzyme Corporation, a global biopharmaceutical company, and subsequently at Sanofi S.A., also a global biopharmaceutical company, following the acquisition of Genzyme by Sanofi in 2011. Additionally, Ms. Lawton previously served two terms as the industry representative on the U.S. Food & Drug Administration's Cell & Gene Therapy Advisory Committee and as Chairman of the Board of the Regulatory Affairs Professional Society. Ms. Lawton currently serves on the boards of directors of ProQR Therapeutics N.V., X4 Pharmaceuticals Inc. and Aeglea Biotherapeutics Inc. Ms. Lawton previously served on the boards of directors of Magenta Therapeutics, Kaleido Biosciences Inc., Verastem, Inc., CoLucid Pharmaceuticals, Inc. prior to its acquisition by Eli Lilly and Cubist Pharmaceuticals, Inc. prior to its acquisition by Merck & Co. Ms. Lawton holds a B.Sc. in pharmacology from Kings College, University of London.

#### **Upcoming Presentations at the 2021 Transplantation and Cellular Therapy (TCT) Annual Meeting**

**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 Oral Presentation:** Monday, February 8, 2021, 2:30 PM CST

**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 Oral Presentation:** Tuesday, February 9, 2021, 3:00 PM CST

**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:** Tuesday, February 9, 2021, 3:00 PM CST

**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 Oral Presentation:** Wednesday, February 10, 2021, 3:00 PM CST

**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

#### **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](http://www.magentatx.com).

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#### **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, including express or implied statements regarding Magenta's future expectations, plans and prospects, including, without limitation, statements regarding expectations and plans for presenting clinical data, projections regarding 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 clinical programs, the timing, progress and success of our collaborations, as well as other statements containing 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 that 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: 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 pre-clinical 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; 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, assumptions and uncertainties regarding the impact of the continuing COVID-19 pandemic on Magenta's business, operations, strategy, goals and anticipated timelines, Magenta's ongoing and planned pre-clinical 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 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.

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