Magenta Therapeutics Announces Update on U.S. FDA Investigational New Drug Application for MGTA-117 in Patients with Acute Myeloid Leukemia and Myelodysplastic Syndrome

July 21, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 21, 2021-- Magenta Therapeutics, Inc. (Nasdaq: MGTA) today announced that it has received a clinical hold letter from the U.S. Food and Drug Administration (FDA) related to its Investigational New Drug Application (IND) filed in June 2021 to initiate a Phase 1/2 clinical trial of MGTA-117 in patients with acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS).

The FDA is requiring that Magenta develop an additional bioassay to be used in conjunction with the PK/PD model to inform dose escalation decisions in addition to safety monitoring. This was the only clinical hold item identified by the FDA and does not relate to the toxicology or manufacturing of MGTA-117. Magenta has initiated the development of the bioassay and intends to work closely with the FDA to determine the application of the bioassay for dose escalation. The FDA has indicated its willingness to continue its engagement on this issue through its "Type A" meeting structure, which Magenta anticipates will help facilitate communication and resolution of the clinical hold.

"We are actively developing the bioassay requested by the FDA and do not expect significant technical challenges in its completion. We expect to request a 'Type A' meeting in the coming weeks and, if successful in resolving this remaining issue, we would anticipate opening the study in Q4 2021," said Jason Gardner, D.Phil., President and Chief Executive Officer, Magenta Therapeutics. "We are greatly appreciative of the FDA's continued engagement as we seek to commence the Phase 1/2 clinical trial of MGTA-117 and develop this potentially first-in-class medicine to improve conditioning options for patients across a number of disease areas."

The Phase 1/2 clinical trial of MGTA-117 is intended as an initial evaluation of its safety, pharmacokinetics (PK) and pharmacodynamics (PD) as a single agent in the relapsed/refractory AML and MDS patient population. Magenta anticipates transitioning the study to transplant-eligible patients after adequate data related to the safety, PK and PD of MGTA-117 have been collected in the relapsed/refractory AML and MDS patient population. Magenta also expects to develop MGTA-117 as a targeted conditioning agent for patients with genetic diseases prior to delivery of *ex vivo* gene therapies.

Magenta ended Q2 2021 with more than $200 million in cash following its recent financing in May 2021 and anticipates being able to execute on its existing plans.

About MGTA-117

Magenta's MGTA-117 program is the Company's lead targeted conditioning product candidate, an antibody-drug conjugate (ADC) designed to selectively deplete hematopoietic stem cells (HSCs) from patients prior to transplant or HSC-based gene therapy to reduce the need for high-dose or high-intensity chemotherapeutic agents or, in the case of gene therapy applications, to potentially eliminate the need for chemotherapeutic agents altogether. MGTA-117 targets the CD117 receptor, which is highly expressed on the cell surface of HSCs and leukemia cells, making it a promising target for conditioning across broad sets of diseases, including certain blood cancers, hemoglobinopathies (sickle cell disease and beta thalassemia) and inherited metabolic disorders.

About Magenta Therapeutics

Magenta Therapeutics is a clinical-stage biotechnology company developing medicines designed to bring the curative power of stem cell transplant to more patients with blood cancers, 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 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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Forward-Looking Statement

This press release may contain forward-looking statements 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 the development of a biomarker assay, working with the FDA to resolve the clinical hold, the anticipated timing of regulatory filings and clinical trials, the transition of the Phase 1/2 trial to transplant-eligible patients, the development of product candidates and advancement of preclinical programs, the potential benefits of our product candidates, as well as other statements containing the words "anticipate," "believe," "continue," "could," "endeavor," "estimate," "expect," "anticipate," "intend," "may," "might," "plan," "potential," "predict," "project," "seek," "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; the development of biomarker assays; 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; discussions with governmental agencies such as the FDA; 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, uncertainties and assumptions regarding the impact of the continuing COVID-19 pandemic on Magenta's business, operations, strategy, goals and anticipated timelines, Magenta's ongoing and planned preclinical 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 are described in additional detail in its Annual Report on Form 10-K filed on March 3, 2021, as updated by Magenta's most recent Quarterly Report 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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