Magenta Therapeutics and Beam Therapeutics Announce Collaboration to Evaluate Targeted Antibody-Drug Conjugate (ADC) MGTA-117 as Conditioning Regimen for Base Editing Therapies

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– MGTA-117 conditioning for precise depletion of cells in the bone marrow could potentially increase patients' benefit from Beam's base editing therapies for sickle cell disease and beta thalassemia –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 15, 2020-- Magenta Therapeutics (Nasdaq: MGTA) and Beam Therapeutics (Nasdaq: BEAM) today announced a non-exclusive research and clinical collaboration agreement to evaluate the potential utility of MGTA-117, Magenta's novel targeted ADC for conditioning of patients with sickle cell disease and beta-thalassemia receiving Beam's base editing therapies. Beam is pursuing two differentiated base editing approaches to treat hemoglobinopathies: its hereditary persistence of fetal hemoglobin (HPFH) program to precisely and robustly elevate fetal hemoglobin, which could be used in treatments for both sickle cell disease and beta-thalassemia, as well as a novel approach to directly correct the sickle causing point mutation (Makassar).

Conditioning is a critical component necessary to prepare a patient's body to receive the edited cells, which carry the corrected gene and must engraft in the patient's bone marrow in order to be effective. Today's conditioning regimens rely on nonspecific chemotherapy or radiation, which are associated with significant toxicities. MGTA-117 precisely targets only hematopoietic stem and progenitor cells, sparing immune cells, and has shown high selectivity, potent efficacy, wide safety margins and broad tolerability in non-human primate models. MGTA-117 may be capable of clearing space in bone marrow to support long-term engraftment and rapid recovery in patients.

Beam has demonstrated the ability to edit individual DNA bases in hematopoietic stem cells at high efficiency and with little impact on the viability of edited cells relative to unedited cells using its novel base editing technology. Combining MGTA-117 with Beam's HPFH and Makassar base editors could meaningfully advance the treatment of patients with sickle cell disease or beta-thalassemia.

"We believe patients will benefit from a more precise process to remove hematopoietic stem cells and prepare them to receive genetic medicines. Magenta has developed targeted ADCs as the preferred modality for our conditioning programs, and we have designed MGTA-117 specifically to optimize it for use with a genetically-modified cell product delivered in a transplant setting," said Jason Gardner, D.Phil., president and chief executive officer, Magenta Therapeutics. "Beam's next-generation base editing technology complements our next-generation conditioning approach very well, and we are excited to combine these strengths to address the still-significant unmet medical needs of the sickle cell and beta-thalassemia patient communities."

"Base editing has the potential to offer lifelong treatment for patients with many diseases, including sickle cell disease and beta-thalassemia. Our novel base editors create precise single base changes in genes without cutting the DNA, enabling durable correction of hematopoietic stem cells with minimal effects on cell viability or genomic integrity," said John Evans, chief executive officer of Beam. "Combining the precision of our base editing technology with the more targeted conditioning regimen enabled by MGTA-117 could further improve therapeutic outcomes for patients suffering from these severe diseases. We look forward to partnering with the Magenta team to explore these novel technologies together."

Beam will be responsible for clinical trial costs related to development of Beam's base editors when combined with MGTA-117, while Magenta will continue to be responsible for all other development costs of MGTA-117. Magenta will also continue to develop MGTA-117 in other diseases, including blood cancers and genetic diseases. Each company will retain all commercial rights to their respective technologies.

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 toxin in-licensed from Heidelberg Pharma. It is designed to precisely deplete only hematopoietic stem and progenitor cells and has shown high selectivity, potent efficacy, wide safety margins and broad tolerability in non-human primate models, suggesting that it may be capable of clearing space in bone marrow to support long-term engraftment and rapid recovery in patients. Magenta plans to complete IND-enabling studies this year and initiate clinical studies in 2021. Magenta will continue to develop MGTA-117 in other diseases, including blood cancers and genetic diseases.

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. Follow Magenta on Twitter: @magentatx.

About Base Editing and Beam Therapeutics

Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company developing precision genetic medicines through the use of base editing. Beam's proprietary base editors create precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This enables a wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization focused on its people, cutting-edge science, and a vision of providing life-long cures to patients suffering from serious diseases. For more information, visit www.Beamtx.com.

Magenta Therapeutics Forward-Looking Statements

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, without limitation, statements regarding the research and clinical collaboration agreement between Magenta and Beam, including the timing, progress and success of the collaboration contemplated under the agreement, the successful evaluation of MGTA-117 in conjunction with Beam's base-editing therapies under the agreement, the anticipated cost allocation and other commercial terms under the agreement, Magenta's strategy and business plan, the future development, manufacture and commercialization between Beam and Magenta as well as statements regarding expectations and plans for the anticipated timing of Magenta's clinical trials and regulatory filings and the development of Magenta's product candidates and advancement of Magenta's preclinical programs. The use of 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 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, risks set forth under the caption "Risk Factors" in Magenta's most recent 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, risks, uncertainties and assumptions regarding the impact of the COVID-19 pandemic to Magenta's business, operations, strategy, goals and anticipated timelines, and risks, uncertainties and assumptions inherent in preclinical and clinical studies, including, without limitation, whether results from preclinical studies or earlier clinical studies will be predictive of the results of future trials and the expected timing of submissions for regulatory approval or review by governmental authorities. 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.

Beam Forward-Looking Statements

This press release contains forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about the timing, progress and success of the collaboration contemplated under the agreement between Beam and Magenta, the successful evaluation of MGTA-117 in conjunction with Beam's base-editing therapies under the agreement, the expected timing of filing INDs applications and the therapeutic applications of Beam's technology. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in development and potential commercialization of our product candidates; Beam's ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from preclinical and clinical trials will be predictive of the results or success of ongoing or later clinical trials; that enrollment of clinical trials may take longer than expected; that Beam's product candidates will experience manufacturing or supply interruptions or failures; that Beam will be unable to successfully initiate or complete the preclinical and clinical development and eventual commercialization of product candidates; that the development and commercialization of Beam's product candidates will take longer or cost more than planned; the impact of COVID-19 on Beam's business and the other risks and uncertainties identified under the heading "Risk Factors" and in Beam's Annual Reports on Form 10-K for the year ended December 31, 2019 and in Beam's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements (except as otherwise noted) speak only as of the date of this press release. Factors or events that could cause Beam's actual results to differ may emerge from time to time, and it is not possible for Beam to predict all of them. Beam undertakes no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

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