Magenta Therapeutics Reports Third Quarter Financial Results and Recent Program Highlights

November 3, 2022

– New clinical results from ongoing MGTA-117 Phase 1/2 clinical trial support earlier observations of target binding, target cell depletion, rapid drug clearance and a favorable tolerability profile; clinical trial has progressed into the third dose escalation cohort; oral presentation of clinical data will be given at the 2022 American Society of Hematology (ASH) meeting in December 2022 –

– CD45 antibody-drug conjugate (ADC) dose-ranging toxicology study successfully completed; IND-enabling studies advancing; program update and development timeline guidance anticipated in December 2022 –

– MGTA-145 stem cell mobilization clinical trial in sickle cell disease patients is enrolling with early clinical data expected in December 2022 –

– Approximately $128.3 million in cash, cash equivalents and marketable securities at the end of Q3 2022; maintains guidance that cash reserves are expected to fund current operating plan into Q2 2024 –

CAMBRIDGE, Mass., Nov. 03, 2022 (GLOBE NEWSWIRE) -- Magenta Therapeutics (Nasdaq: MGTA), a clinical-stage biotechnology company developing novel medicines designed to bring the curative power of stem cell transplant to more patients, today reported financial results for the third quarter ending September 30, 2022, and recent program highlights.

"We are building momentum in the MGTA-117 clinical trial with new clinical results and are making progress across our pipeline, including our second targeted conditioning program CD45-ADC," said Jason Gardner, President and Chief Executive Officer of Magenta Therapeutics, Inc. "In the MGTA-117 Phase 1/2 clinical trial, we continue to enroll and generate clinical data in higher-dose cohorts. Our preclinical work has been particularly helpful in predicting our clinical experience in these early dose cohorts regarding MGTA-117 activity at different exposure levels and the overall tolerability profile. We look forward to presenting available clinical data at ASH and using our data to support interactions with regulators as we plan to advance MGTA-117 to stem cell transplant-eligible patients and into gene therapy."

Program Highlights:

MGTA-117 Phase 1/2 Clinical Trial Progression and Data Disclosure Expectations

MGTA-117 is Magenta's most advanced targeted conditioning product candidate designed to deplete target cells prior to a patient undergoing stem cell transplant or receiving an ex vivo gene therapy product. The program is currently enrolling patients with relapsed/refractory acute myeloid leukemia (AML), and myelodysplastic syndromes (MDS), in a Phase 1/2 dose escalation clinical trial. MGTA-117 is an anti-CD117 antibody conjugated to an amanitin payload. CD117, also known as c-Kit receptor, is highly expressed on hematopoietic stem cells, progenitor cells, and leukemic cells.

- *Enrollment Progress.* Magenta has completed enrollment in Cohort 1 and Cohort 2. In addition, Magenta has enrolled a sufficient number of patients to complete Cohort 3, provided that the patients complete their respective dose-limiting toxicity (DLT) observation periods. No serious adverse events have been deemed to be related to MGTA-117, and no dose-limiting toxicities have been observed to date. Our clinical experience has confirmed that patients with relapsed/refractory AML are at high risk for multiple disease complications, including susceptibility to infection, all of which can rapidly progress at any time leading to severe morbidity or mortality. All enrolled patients have contributed data to the clinical trial independent of the completion of the DLT period.
- *Proof-of-Mechanism*. Available clinical data, including new data since the ASH abstract submission in August 2022, support proof-of-mechanism for MGTA-117 due to evidence of its ability to bind CD117-expressing cells, deplete CD117-expressing cells, clear the body rapidly and be well-tolerated.
- *Regulatory Plans*. Magenta has initiated requests for formal engagement with multiple regulatory authorities for the purpose of transitioning the clinical program into transplant-eligible AML and MDS patients. The pending regulatory interactions are expected to focus on MGTA-117's clinical data relating to target binding, drug clearance and stability and tolerability across multiple dose levels. All available clinical data will be used to support these regulatory interactions, as well as the predictive preclinical modeling in non-human primates that has closely matched our clinical experience to date.
- *Gene Therapy*. Magenta expects data from the Phase 1/2 trial to also inform clinical development planning and enable regulatory engagements for MGTA-117 as a potential monotherapy prior to patients undergoing autologous *ex vivo* gene therapy. Magenta has existing clinical collaborations with gene therapy companies and anticipates entering into additional collaborations as data progresses.
- *ASH Presentations*. As disclosed separately, Magenta will present clinical and preclinical data at the American Society of Hematology (ASH) Annual Meeting in December 2022. The MGTA-117 clinical presentation will include data on pharmacokinetics, pharmacodynamic activity and the tolerability of MGTA-117. In light of the need to collect and finalize complete Cohort 3 data, Magenta currently anticipates presenting Cohort 1 and Cohort 2 clinical data at ASH. Any data not included in the ASH presentation is anticipated to be available at a scientific conference in Q1 2023.

CD45-Antibody Drug Conjugate (ADC): Second Targeted Conditioning Program

Magenta's CD45-ADC is designed to selectively target and deplete both stem cells and immune cells and is intended to replace the use of chemotherapy and radiation-based conditioning prior to stem cell transplant in patients with blood cancers and autoimmune diseases.

- Magenta has completed a dose-ranging toxicology preclinical study successfully with no unexpected findings. The data inform dosing for a Good Laboratory Practices toxicology study intended to support a planned Investigational New Drug (IND) application.
- Manufacturing and other IND-enabling activities are ongoing, and Magenta is preparing for regulatory interactions and clinical development activities.
- Magenta expects to provide a further update on the CD45-ADC program in December 2022.

MGTA-145 Stem Cell Mobilization and Collection
Magenta is developing MGTA-145, in combination with plerixafor, to improve the process by which stem cells are released out of the bone marrow and into the bloodstream, known as stem cell mobilization. The mobilized cells are then collected and available for transplant. This is the first step for patients and is required for the majority of transplants and stem cell gene therapies.

- Magenta, in partnership with bluebird bio, has initiated a Phase 2 clinical trial in sickle cell disease to evaluate the utility of MGTA-145, in combination with plerixafor, for the mobilization and collection of stem cells in patients with sickle cell disease. Mobilization and collection are difficult in sickle cell disease patients where granulocyte colony-stimulating factor (GCSF) cannot be used, and there is a clear unmet medical need.
- Magenta anticipates generating initial data from this clinical trial in December 2022 followed by a more comprehensive data set in H1 2023.

Financial Results:
Cash Position: Cash, cash equivalents and marketable securities as of September 30, 2022, were $128.3 million, compared to $176.9 million as of December 31, 2021. The September cash balance includes gross proceeds of $3.0 million from our “at-the-market” facility. Magenta anticipates that its cash, cash equivalents and marketable securities will be sufficient to fund its current operational plan into Q2 2024.

Research and Development Expenses: Research and development expenses were $11.2 million in the third quarter of 2022, compared to $10.8 million in the third quarter of 2021. The increase was driven primarily by higher preclinical and manufacturing costs to support our IND-enabling studies for CD45-ADC, offset by a decrease in clinical trial costs related to our mobilization program.

General and Administrative Expenses: General and administrative expenses were $6.1 million for the third quarter of 2022, compared with $7.5 million in the third quarter of 2021. The decrease was primarily due to a decrease in stock-based compensation.

Net Loss: Net loss was $16.1 million for the third quarter of 2022, compared to net loss of $17.4 million for the third quarter of 2021.

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 to revolutionize blood and immune reset to allow more patients to take advantage of the curative potential of stem cell transplant and potentially improve eligibility for future gene therapies.

Magenta is based in Cambridge, Mass. For more information, please visit www.magentatx.com.

Follow Magenta on Twitter: @magentatx.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, as amended. These statements include, without limitation, implied and express statements relating to: Magenta’s future business expectations, plans and prospects; the potential of, and expectations for, Magenta’s product candidate pipeline; the potential benefits and expected performance of Magenta’s product candidates and programs; the development of product candidates and advancement of preclinical and clinical programs, including, without limitation, patient enrollment; expectations, plans and timing for preclinical activities, clinical trials and related results involving Magenta’s product candidates; expectations, plans and timing for the generation, receipt and disclosure of preclinical and clinical trial data, toxicology results, and other results involving Magenta’s product candidates; timing for the disclosure of developmental timelines, developmental plans and program updates regarding Magenta’s product candidates; timelines and expectations for dosing, dosing regimens and administration; the use of data to support interactions with regulators as Magenta plans to advance MGTA-117 to stem cell transplant-eligible patients and into gene therapy; the completion of dose-limiting toxicity observation periods; regulatory interactions; the planned transition of the MGTA-117 Phase 1/2 clinical trial into transplant-eligible AML and MDS patients; expectations that regulatory interactions will focus on MGTA-117’s clinical data relating to target binding, drug clearance and stability and tolerability across multiple dose levels; the planned use of available clinical data to support regulatory interactions, as well as the predictive preclinical modeling in non-human primates that has closely matched Magenta’s clinical experience; expectations that data from the Phase 1/2 trial will inform clinical development planning and enable regulatory engagements for MGTA-117 as a potential monotherapy prior to patients undergoing autologous ex vivo gene therapy; the predictive value of Magenta’s MGTA-117 preclinical modeling; whether present results will collectively inform the continued development of MGTA-117; the anticipation of entering into additional collaborations as data progresses; that data from a dose-ranging toxicology preclinical study for CD45-ADC inform dosing for a Good Laboratory Practices toxicology study intended to support a planned Investigational New Drug application; the expectation that clinical data from additional dose-escalation cohorts will support Magenta’s prior clinical observations and data in the MGTA-117 Phase 1/2 clinical trial in patients with relapsed/refractory AML and MDS; the anticipated benefits of Magenta’s revised operating plan; and Magenta’s current anticipation and guidance regarding the ability of its cash, cash equivalents and marketable securities to fund its current operating plan into Q2 2024.

Words such as “anticipate,” “believe,” “continue,” “could,” “designed,” “endeavor,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “preliminary,” “will,” “would” and similar expressions are intended 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: volatility and uncertainty in the capital markets for biotechnology companies; uncertainties inherent in preclinical and clinical trials and in the availability and timing of data from ongoing and planned clinical and preclinical trials; the ability to

initiate, enroll, conduct or complete ongoing and planned preclinical and clinical trials; vulnerability and/or fragility of, and the presence of underlying disorders in, the patient population for the clinical trials of Magenta's product candidates, including the MGTA-117 Phase 1/2 clinical trial in patients with relapsed/refractory AML and MDS; the delay of any current or planned preclinical or clinical trials, or the delay in development of Magenta's product candidates; whether results from preclinical or earlier clinical trials will be predictive of the results of future trials; interactions with regulatory agencies such as the U.S. Food and Drug Administration; the expected timing of submissions for regulatory approval to conduct or continue trials or to market products; Magenta's ability to successfully demonstrate the safety and efficacy of its product candidates; whether Magenta's cash resources will be sufficient to fund Magenta's foreseeable and unforeseeable operating expenses and capital expenditure requirements; and risks, uncertainties and assumptions regarding the impact of the continuing COVID-19 pandemic on Magenta's business, operations, preclinical activities, clinical trials, strategy, goals and anticipated timelines. These and other risks are described in additional detail in Magenta's Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, expected to be filed on or about November 3, 2022, and its other filings made with the Securities and Exchange Commission from time to time. Any forward-looking statements contained in this press release represent Magenta's views only as of today and should not be relied upon as representing its views as of any subsequent date. Magenta explicitly disclaims any obligation to update any forward-looking statements, except to the extent required by law.

Contact:
Jill Bertotti, Real Chemistry (advisor to Magenta)
714-225-6726
jbertotti@realchemistry.com

Magenta Therapeutics, Inc.

STATEMENTS OF OPERATIONS
(unaudited)
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses:				
Research and development	$ 11,201	$ 10,795	$ 39,351	$ 33,652
General and administrative	6,052	7,450	19,819	20,900
Total operating expenses	17,253	18,245	59,170	54,552
Loss from operations	(17,253)	(18,245)	(59,170)	(54,552)
Interest and other income, net	1,190	818	2,886	2,708
Net loss	$ (16,063)	$ (17,427)	$ (56,284)	$ (51,844)
Net loss per share, basic and diluted	$ (0.27)	$ (0.30)	$ (0.95)	$ (0.97)
Weighted average common shares outstanding, basic and diluted	59,269,965	58,583,476	58,963,280	53,655,314

BALANCE SHEET DATA
(unaudited)
(In thousands)

	September 30, 2022	**December 31, 2021**
Cash, cash equivalents and marketable securities	$ 128,284	$ 176,926
Working capital	119,176	169,830
Total assets	164,123	189,934
Stockholders' equity	124,025	172,672

Source: Magenta Therapeutics