



## Magenta Therapeutics Reports First Quarter Financial Results, Early Clinical Observations from MGTA-117 Clinical Trial and Other Program Highlights

May 16, 2022

- Clinical observations from preliminary data at the lowest dose of MGTA-117 in a Phase 1/2 clinical trial indicate target binding, reduction of target cells in bone marrow, rapid drug clearance and a favorable tolerability profile –
- IND-enabling preclinical studies ongoing for CD45 antibody-drug conjugate; key dose-ranging toxicology results expected in 2H 2022 –
- Startup activities progressing for MGTA-145 stem cell mobilization in sickle cell disease clinical trial; initial data anticipated in 2H 2022 –
- Ended Q1 2022 with approximately \$156.6 million in cash, cash equivalents and marketable securities; maintains guidance that cash reserves are expected to fund operating plan into Q2 2024 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 16, 2022-- 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 first quarter ending March 31, 2022, early clinical observations from its MGTA-117 clinical trial and other recent program highlights.

"We are pleased with the progress of our MGTA-117 Phase 1/2 clinical trial. Our clinical observations from preliminary data indicate that MGTA-117 is functioning as designed by binding to the intended cells with a post-dose reduction of target cells in the bone marrow, clearing the body rapidly and doing so with a favorable tolerability profile. With this level of measurable activity at our lowest dose, we believe we will collect enough information in 2022 from the next 1-2 cohorts to build a data set for communications with regulators for our planned transition to the transplant-eligible AML patient population. In light of the turbulence in the capital markets, we are also pleased to have a strong cash position and a projected lower quarterly spending rate which we believe will allow us to reach our critical value inflection points, including possible proof-of-concept of MGTA-117 in transplant-eligible AML patients and genetic diseases with gene therapy," said Jason Gardner, President and Chief Executive Officer of Magenta Therapeutics.

### Program Highlights:

#### **MGTA-117 Phase 1/2 Clinical Trial Ongoing**

Clinical Trial Design and Objectives. Magenta is conducting a Phase 1/2 dose-escalation clinical trial of MGTA-117 in patients with relapsed/refractory acute myeloid leukemia (AML) and myelodysplastic syndrome with excess blasts (MDS-EB).

- Clinical Trial Status and Scope of Early Clinical Observations. The clinical trial has progressed from Cohort 1 to Cohort 2 after meeting all protocol requirements for dose escalation. Patients in Cohort 1, as expected, had varying degrees of disease burden as measured in part by the percentage of leukemic cells in the bone marrow and the bloodstream. Clinical observations were made from a preliminary data set from Cohort 1 which utilizes the trial's lowest dose of MGTA-117 (0.02 mg/kg). Three patients with relapsed/refractory AML completed the safety evaluation period. A fourth patient with high disease burden did not complete the evaluation period due to disease progression with no drug-related adverse events. Cohort 2 is now open and enrolling and MGTA-117 will be evaluated as a single dose of 0.04 mg/kg.
- Early Clinical Observations from Cohort 1.
  - Target Engagement/Binding: Preliminary data from four patients were available for measuring target engagement. In all patients, MGTA-117 was shown to bind CD117<sup>+</sup> cells in the blood as measured by a receptor occupancy assay.
  - Cell Depletion: Bone marrow aspirates were obtained for three patients at baseline and post-dosing and provided evidence supportive of biologic activity.
    - Reductions of Erythroid Progenitor Cells in the Bone Marrow: One patient had measurable reductions of CD117<sup>+</sup> erythroid progenitor cells in the bone marrow following MGTA-117 administration.
    - Reductions of Blasts in the Bone Marrow: An additional patient had an approximate 83% reduction of blasts in the bone marrow at day 14 post-dosing (from 6% to 1%). The patient proceeded to a conditioning regimen followed by stem cell transplant. The patient's baseline profile closely resembled that of transplant-eligible AML patients due to relatively low percentages of blasts in the bone marrow and bloodstream. Magenta believes that this outcome provides an encouraging early signal in support of MGTA-117's planned transition to the transplant-eligible AML patient population.
  - Rapid Drug Clearance: Preliminary data from four patients were available for measuring drug clearance. In all patients, MGTA-117 was deemed to be cleared 48 hours after dosing. Rapid and sufficient clearance of conditioning agents from circulation is a necessary step before stem cell infusion.
  - Favorable Tolerability Profile: For all four patients, no unexpected or serious drug-related adverse events were reported, no dose-limiting toxicities were observed and no drug-related adverse events higher than Grade 1 were reported.

"We are encouraged by these preliminary data. Post-dose reduction of progenitor and tumor blast cell populations in bone marrow suggests biologic

activity. We anticipate that dose escalation will lead to further drug activity and enable identification of an appropriate dose for development in patients eligible for transplant,” said Dr. Jeff Humphrey, Chief Medical Officer of Magenta Therapeutics.

- **MGTA-117 Clinical Data Disclosure Expectations in 2022.** Magenta expects to report additional progress updates and clinical observations from multiple dose-escalation cohorts in 2022, including providing patient-level data from this clinical trial at a scientific congress later this year.
- **Plans to Transition to Transplant-Eligible Patients.** The company anticipates that further data from the current clinical trial showing MGTA-117 at high receptor occupancy levels with well-tolerated cell depletion in the blood and/or bone marrow will be supportive of the planned transition to transplant-eligible patients. Magenta is planning to engage with regulatory authorities prior to amending the clinical trial protocol to evaluate MGTA-117 as a targeted conditioning agent in combination with reduced intensity chemotherapy prior to a stem cell transplant. Simultaneously with the planned clinical trial transition, Magenta expects to initiate clinical collaboration planning with existing and potential gene therapy partners to explore MGTA-117 as a single agent conditioning regimen prior to autologous stem cell gene therapy.

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

*CD45 is broadly expressed on hematopoietic cells and Magenta’s CD45-ADC is designed to selectively target and deplete both stem cells and lymphocytes, which would enable patients with blood cancers and autoimmune diseases to avoid the use of chemotherapy prior to stem cell transplant.*

- Magenta has initiated IND-enabling studies with data from a key dose-ranging toxicology study expected in the second half of 2022.

#### **MGTA-145 Stem Cell Mobilization and Collection**

*Magenta is developing MGTA-145, in combination with plerixafor, to improve stem cell mobilization from bone marrow into the bloodstream. Collection of peripheral blood stem cells, known as stem cell mobilization, is a common source of stem cells for hematopoietic stem cell transplants and gene therapy applications.*

- Magenta is preparing for the initiation of a Phase 2 clinical trial in sickle cell disease (SCD) in collaboration with bluebird bio to evaluate MGTA-145, in combination with plerixafor, for mobilization and collection of stem cells in patients with sickle cell disease. Mobilization and collection are difficult in sickle cell disease, and there is a clear unmet medical need. Initial data from this trial are expected in the second half of 2022.

#### **Financial Results:**

**Cash Position:** Cash, cash equivalents and marketable securities as of March 31, 2022, were \$156.6 million, compared to \$176.9 million as of December 31, 2021. Magenta anticipates that its cash, cash equivalents and marketable securities will be sufficient to fund its current operational plan into the second quarter of 2024.

**Research and Development Expenses:** Research and development expenses were \$16.5 million in the first quarter of 2022, compared to \$11.7 million in the first quarter of 2021. The increase was driven primarily by the achievement of a development milestone under Magenta’s license agreement related to MGTA-117, an increase in preclinical and manufacturing costs to support our CD45-ADC IND enabling studies, offset by a decrease in costs related to MGTA-456 which was discontinued. The increase was also due to an increase in research and development headcount.

**General and Administrative Expenses:** General and administrative expenses were \$7.3 million for the first quarter of 2022, compared to \$7.0 million for the first quarter of 2021.

**Net Loss:** Net loss was \$23.0 million for the first quarter of 2022, compared to net loss of \$17.5 million for the first 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 immune and blood reset to allow more patients to take advantage of the curative potential of stem cell transplant as well as potentially improve eligibility for future gene therapies.

Magenta is based in Cambridge, Mass. For more information, please visit [www.magentatx.com](http://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; expectations, plans and timing for preclinical activities, clinical trials and related results involving Magenta’s product candidates; timing for the receipt and disclosure of preclinical and clinical data, clinical toxicology results, and other results involving Magenta’s product candidates; timelines and expectations for patient dosing, dosing regimens and administration; the collection of enough information in 2022 from the next 1-2 cohorts of the MGTA-117 Phase 1/2 clinical trial to build a data set for communications with regulators for the planned transition to the transplant-eligible AML patient population; the anticipated benefits of Magenta’s revised operating plan; the belief that Magenta’s cash position will allow it to reach critical value inflection points, including

possible proof-of-concept of MGTA-117 in transplant-eligible AML patients and genetic diseases with gene therapy; the belief that preliminary data from Cohort 1 of the MGTA-117 Phase 1/2 clinical trial provide an encouraging early signal in support of MGTA-117's planned transition to the transplant-eligible AML patient population; the anticipation that dose escalation will lead to further drug activity and enable identification of an appropriate dose for development in patients eligible for transplant; the anticipation that further data from the current clinical trial showing MGTA-117 at high receptor occupancy levels with well-tolerated cell depletion in the blood and/or bone marrow will be supportive of the planned transition to transplant-eligible patients; plans to engage with regulatory authorities prior to amending the clinical trial protocol to evaluate MGTA-117 as a targeted conditioning agent in combination with reduced intensity chemotherapy prior to a stem cell transplant; the expectation that Magenta will initiate clinical collaboration planning with existing and potential gene therapy partners to explore MGTA-117 as a single agent conditioning regimen prior to autologous stem cell gene therapy; preparations for the initiation of a Phase 2 clinical trial in sickle cell disease (SCD) in collaboration with bluebird bio to evaluate MGTA-145, in combination with plerixafor, for mobilization and collection of stem cells in patients with sickle cell disease; and Magenta's current anticipation 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 acute myeloid leukemia and myelodysplasia-excess blasts; the delay of any current or planned preclinical or clinical trials or the 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 March 31, 2022, expected to be filed on or about May 16, 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.

#### **Magenta Therapeutics, Inc.**

#### **STATEMENTS OF OPERATIONS**

(unaudited)

(In thousands, except share and per share data)

	<b>Three Months Ended March 31,</b>	
	<b>2022</b>	<b>2021</b>
Operating expenses:		
Research and development	\$ 16,547	\$ 11,728
General and administrative	7,287	6,969
Total operating expenses	23,834	18,697
Loss from operations	(23,834)	(18,697)
Interest and other income, net	884	1,208
Net loss	\$ (22,950)	\$ (17,489)
Net loss per share, basic and diluted	\$ (0.39)	\$ (0.36)
Weighted average common shares outstanding, basic and diluted	58,799,157	48,567,106

#### **BALANCE SHEET DATA**

(unaudited)

(In thousands)

	<b>March 31, 2022</b>	<b>December 31, 2021</b>
Cash, cash equivalents and marketable securities	\$ 156,604	\$ 176,926
Working capital	146,146	169,830
Total assets	194,385	189,934
Stockholders' equity	151,188	172,672

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