Magenta Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results and Recent Business Highlights

March 3, 2020

– New MGTA-117 antibody-drug conjugate (ADC) clinical candidate for conditioning demonstrated broad therapeutic index in data highlighted in oral presentation at Transplant and Cellular Therapies (TCT) conference; advancing MGTA-117 to generate clinical data in 2021–

–Reported first-ever successful gene therapy transplant of non-human primates with targeted single-agent CD117-ADC with no chemotherapy at ASH annual meeting and in best abstract at TCT–

– Completed dosing in Phase 1 MGTA-145 trial, demonstrating rapid, single-day first line stem cell mobilization and collection; met all primary and secondary endpoints and presented data in oral presentation at TCT–

–Presented first preclinical immune reset data with CD45-ADC at the American College of Rheumatology (ACR) annual meeting –

– Presented additional data from Phase 2 study of MGTA-456 showing clinically meaningful durable benefits for patients with inherited metabolic disorders at TCT–

–Ended year with $145.7 million in cash, cash equivalents and marketable securities –

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Magenta Therapeutics (NASDAQ: MGTA), a clinical-stage biotechnology company developing novel medicines to bring the curative power of immune reset to more patients, today reported financial results for the fourth quarter and full year ended December 31, 2019 and recent business highlights.

“2019 was a year marked by crucial progress towards our vision of immune reset, including the advancement of our two lead conditioning programs and our two clinical programs. We generated unprecedented data from our ADC-based targeted conditioning platform, and we are particularly pleased with our new MGTA-117 clinical candidate for targeted conditioning for stem cell transplant or gene therapy. Results presented last month at the TCT conference highlighted the potency, safety and broad therapeutic index of MGTA-117, well above that of currently approved ADCs at this stage of development. We look forward to moving this program into the clinic with initial clinical data expected in 2021,” said Jason Gardner, D.Phil., President and Chief Executive Officer, Magenta. “We also presented updated clinical data for our first-line stem cell mobilization program, MGTA-145. We have completed dosing in the Phase 1 trial and are moving forward with multiple Phase 2 studies this year. We are developing MGTA-145 as the new standard of care for first line stem cell mobilization and immune system rebuild with the potential to benefit all of the patients eligible for transplant each year.”

Recent Business Highlights:

New MGTA-117 ADC clinical candidate for conditioning demonstrates broad therapeutic index; advancing MGTA-117 to generate patient clinical data in 2021: Magenta presented new data at the TCT conference in February 2020 demonstrating that MGTA-117’s chemically modified linker-toxin between antibody and payload resulted in potent depletion of stem and progenitor cells with an improved therapeutic index over prior molecules: potency ratio of 30 fold (therapeutic index; typical range for approved ADCs at this stage of development is two to six fold). MGTA-117 was developed under a partnership with Heidelberg Pharma that grants Magenta exclusive worldwide development and commercialization rights for ADCs using an amanitin payload and targeting CD117. The antibody and payload are advancing in GMP manufacture. Magenta is scaling up manufacturing of MGTA-117 and completing IND-enabling studies in 2020. The Company intends to move this new product candidate into the clinic with initial clinical data expected in 2021.

Reported first-ever successful gene therapy transplant of non-human primates with targeted single-agent CD117-ADC with no chemotherapy: Data presented at the American Society of Hematology (ASH) annual meeting in December 2019, showed the first-ever successful transplant of gene-modified cells in non-human primates using a tool molecule CD117-targeted, single-agent ADC, without the use of chemotherapy or radiation. These landmark results validate and advance Magenta’s conditioning platform.

Completed dosing in Phase 1 MGTA-145 trial, demonstrating rapid, single-day first line stem cell mobilization and collection; met all primary and secondary endpoints: At TCT, Magenta presented data from the Phase 1 trial of MGTA-145 in healthy volunteers. Data showed that MGTA-145 was safe and well tolerated as a single agent and in combination with plerixafor and demonstrated rapid, single-day mobilization and collection of sufficient numbers of stem cells. The Company has completed dosing in the Phase 1 trial and intends to move this program into multiple Phase 2 trials in patients in 2020. The Phase 2 trials will include both allogeneic and autologous transplant settings and will evaluate mobilization and collection of functional cells and engraftment of the cells after transplant to rebuild the immune system.

Presented first preclinical immune reset data with CD45-ADC at ACR: In November 2019, Magenta presented the first data on the use of targeted ADCs to reset the immune system and halt progression of autoimmune disease. Results showed that a single dose of CD45-ADC removed disease-causing cells, enabled successful reset and rebuild of the immune system and was well tolerated in models of multiple sclerosis, systemic sclerosis and inflammatory arthritis. Further, a single dose of CD45-ADC significantly delayed disease onset in a model of multiple sclerosis that has successfully provided preclinical proof of concept for clinically validated standard of care therapies. Magenta has identified a lead antibody and has progressed this program into IND-enabling studies, which the Company plans to further advance in 2020. On November 11, 2019, Magenta announced that it had exercised its option with Heidelberg Pharma for exclusive worldwide development and marketing rights for ADCs using an amanitin payload and targeting CD45.

Presented additional data from Phase 2 study of MGTA-456 showing clinically meaningful durable benefits for patients with inherited metabolic disorders: In updated results presented at TCT, two patients with cerebral adrenoleukodystrophy treated with MGTA-456 in the Phase 2

study in inherited metabolic disorders showed early and durable resolution of disease at one year of follow-up, as measured by resolution of brain inflammation on MRI. The two patients also had stable Loes and neurological function scores, consistent with a halt in disease progression. Patients with Hurler syndrome showed normalized levels of blood a-L-iduronidase and had decreased levels of Hurler-specific urine glycosaminoglycans, the toxic metabolites implicated in disease. Magenta intends to complete enrollment in the Phase 2 trial in 2020 and continue dialogue with the FDA under the RMAT designation on design of a registration-enabling study, and to have discussions with the European Medicines Agency for development in Europe.

Appointed Chief People Officer and SVP of Manufacturing: In February, Magenta announced that it had expanded its senior leadership with two new strategic hires, Kristen Stants as Chief People Officer and Li Malmberg, Ph.D., as Senior Vice President, Head of Manufacturing. Ms. Stants is a seasoned human resources professional who joined Magenta from Alexion Pharmaceuticals, where she served as Head of Talent Strategy, responsible for organizational development and talent acquisition to expand the company's therapeutic pipeline. Dr. Malmberg is an accomplished technical leader with more than 25 years of manufacturing experience, coming to Magenta from Celgene Corporation, where she served as Vice President, Head of Biologics Development and Manufacturing, responsible for the company's manufacturing development and biologics manufacturing organization and advanced more than 20 biologics molecule and launched one commercial product.

Financial Results:

Cash Position: Cash, cash equivalents and marketable securities as of December 31, 2019, were $145.7 million, compared to $142.6 million on December 31, 2018. Magenta anticipates that its cash, cash equivalents and marketable securities will be sufficient to fund operations and capital expenditures into the fourth quarter of 2021.

Research and Development Expenses: Research and development expenses were $18.7 million in the fourth quarter of 2019, compared to $12.4 million in the fourth quarter of 2018. The increase was driven primarily by investments in manufacturing related to our conditioning programs and MGTA-456, increases in personnel to support a clinical-stage company, as well as clinical activities for MGTA-145.

General and Administrative Expenses: General and administrative expenses were $5.9 million for the fourth quarter of 2019, compared to $5.5 million for the fourth quarter of 2018. The increase was primarily due to an increase in personnel and facilities associated with the growth of the Company.

Net Loss: Net loss was $23.2 million for the fourth quarter of 2019, compared to net loss of $16.7 million for the fourth quarter of 2018.

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.

Forward-Looking Statement
This press release may contain forward-looking statements, including express or implied statements regarding Magenta's future expectations, plans and prospects, including, without limitation, statements regarding expectations and plans for presenting pre-clinical and clinical data, projections regarding future revenues and financing performance, 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 preclinical programs, as well as other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," might," "plan," "potential," "project," "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; whether interim results from a clinical trial will be predictive of the final results of the trial; 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, including review under accelerated approval processes; orphan drug designation eligibility; 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; and other risks concerning Magenta's programs and operations are described in additional detail in its registration statement on Form S-1, its Annual Report on Form 10-K filed on March 19, 2019, its Quarterly Reports 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.

Magenta Therapeutics, Inc.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2019	**2018**	**2019**	**2018**
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				

Research and development	18,714	12,390	59,208	41,340
General and administrative	5,923	5,540	23,761	18,623
Total operating expenses	24,637	17,930	82,969	59,963
Loss from operations	(24,637)	(17,930)	(82,969)	(59,963)
Interest and other income, net	1,400	1,251	6,200	2,448
Net loss	(23,237)	(16,679)	(76,769)	(57,515)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(88)
Net loss attributable to common stockholders	$ (23,237)	$ (16,679)	$ (76,769)	$ (57,603)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.59)	$ (0.50)	$ (2.07)	$ (3.13)
Weighted average common shares outstanding, basic and diluted	39,068,523	33,204,929	37,014,875	18,389,576

BALANCE SHEET DATA
(unaudited)
(In thousands)

	December 31,	
	2019	**2018**
Cash, cash equivalents and marketable securities	$ 145,729	$ 142,570
Working capital	135,728	134,902
Total assets	161,514	157,313
Stockholders' equity	141,193	145,648

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