# Magenta Therapeutics Announces First Patient Transplanted with MGTA-456 in Phase 2 Study in Inherited Metabolic Disorders

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**Cambridge, Mass.** – April 5, 2018 – Magenta Therapeutics, a biotechnology company developing novel medicines to bring the curative power of bone marrow transplant to more patients, today announced treatment of the first patient with an inherited metabolic disorder in a Phase 2 study of MGTA-456, an expanded cord blood stem cell product. MGTA-456 is a first-in-class allogeneic stem cell therapy consisting of a single umbilical cord blood unit expanded with an aryl hydrocarbon receptor (AHR) antagonist then administered to a patient through a bone marrow transplant.

“Inherited metabolic disorders can lead to disability and early death in affected children. Often bone marrow transplantation is the preferred or only available therapy. Cord blood transplantation is commonly the best option, but it remains a difficult therapy and may lead to life-threatening complications,” said Paul Orchard, M.D., Medical Director of the Inherited Metabolic & Storage Disease Bone Marrow Transplantation Program, University of Minnesota. “We believe transplantation utilizing a larger number of stem cells with MGTA-456 may reduce risk and enhance recovery post transplant, thereby providing these children and their families the best opportunity to achieve an optimal outcome.”

“MGTA-456 has demonstrated clinical proof of concept in 36 patients with blood cancers, and we are now exploring its potential in patients with inherited metabolic diseases,” said John Davis, M.D., M.P.H., chief medical officer, Magenta Therapeutics. “Because of the ability of MGTA-456 to significantly expand a single cord blood unit into higher cell doses, which increases the likelihood and speed of engraftment, we believe it will represent a compelling treatment option for these patients. We intend to investigate MGTA-456 in other debilitating diseases where we believe it could deliver transformative benefit to patients.”

The Phase 2 study of MGTA-456 is designed to enroll 12 patients with Hurler’s syndrome, adrenoleukodystrophy, metachromatic leukodystrophy or globoid cell leukodystrophy. The primary endpoint is engraftment after transplantation and the secondary endpoint is transplant-related safety and tolerability. The study is currently enrolling patients at the University of Minnesota and may expand to other sites in the future. More information on the study can be found at https://clinicaltrials.gov/ct2/show/NCT03406962.

**About MGTA-456**

Our most advanced product candidate, MGTA-456, is a first-in-class allogeneic stem cell therapy consisting of a single umbilical cord blood unit expanded with an aryl hydrocarbon receptor (AHR) antagonist. AHR antagonism is a novel, well-studied and clinically validated pathway that controls cell self-renewal and differentiation. MGTA-456 has the potential to allow patients to be treated with higher cell doses than would otherwise be possible, and to have access to better HLA-matched cord blood units, both of which have been shown to provide better outcomes and lower rates of post-transplant complications. MGTA-456 is currently being studied in a Phase 2 clinical trial in patients with inherited metabolic disorders.

**About Bone Marrow Transplant**

Healthy bone marrow stem cells and the blood cells they create are crucial for survival, but certain diseases can affect the bone marrow, interfering with its ability to function properly. A bone marrow transplant is a process to replace unhealthy bone marrow with healthy bone marrow stem cells. Bone marrow transplant can save the lives of patients with blood cancers and genetic diseases and is a potential cure for patients with severe refractory autoimmune diseases. Currently bone marrow transplant is still associated with risk, toxic side effects, and complexity for patients that could benefit from the procedure. Magenta is working to address these challenges through its integrated portfolio of therapeutics.

**About Magenta Therapeutics**

Magenta Therapeutics is a biotechnology company developing therapeutics to revolutionize bone marrow transplant for patients with autoimmune diseases, blood cancers and genetic diseases. By creating a platform focused on critical areas of unmet need, Magenta Therapeutics is pioneering an integrated approach to extend the curative power of bone marrow transplant to more patients, by making the process more effective, safer, and easier. Founded by internationally recognized leaders in bone marrow transplant medicine, Magenta Therapeutics was launched in 2016 by Third Rock Ventures and Atlas Venture and is headquartered in Cambridge, Mass. For more information, please visit www.magentatx.com.

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