Cartesian Initiates CAR-T Clinical Trial in Myasthenia Gravis

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First CAR-T to enter clinical development for an autoimmune disease

Descartes-08 engineered to have a defined, predictable half-life, enabling repeat dosing to maximize potential for potency while minimizing risk of toxicity

Gaithersburg, MD, November 5, 2019 – Cartesian Therapeutics, a fully integrated, clinical-stage biopharmaceutical company developing cell and gene therapies for cancer and autoimmune diseases, today announced that it has initiated a Phase 1/2 clinical trial (NCT04146051) of its lead CAR-T candidate, Descartes-08, in patients with generalized myasthenia gravis (GMG). To the company's knowledge, the program is the first CAR-T investigational candidate to enter clinical development for an autoimmune disease.

"Patients with severe GMG have limited treatment options and are often dependent on nonselective, chronic immunosuppressive therapies (ISTs) that have long-term toxicities," said Volkan Granit, MD, the trial's principal investigator and Assistant Professor of Neurology at the University of Miami Miller School of Medicine. "Cartesian's CAR-T technology selectively targets the primary culprit in the disease: antibody-producing plasma cells. Such selective targeting would be a first in GMG and could help patients discontinue use of chronic ISTs."

"An increasing trend in the treatment of myasthenia gravis is the development of more targeted therapies," said Michael Benatar, MD, PhD, Professor of Neurology, Chief of the Neuromuscular Division at the University of Miami Miller School of Medicine, and co-investigator on the study. "The strategy of targeting for elimination the aberrant long-lived plasma cells that produce these autoantibodies with CAR-T cells is a novel and very promising approach."

Descartes-08 is a CD8+ CAR-T investigational therapy that targets cells expressing B-cell Maturation Antigen (BCMA), a protein expressed by all plasma cells. "Unlike conventional CAR-T, which has the potential for uncontrolled proliferation and ensuing severe toxicity, Descartes-08 is engineered to have a defined and predictable half-life, enabling repeat dosing to maximize potency while minimizing risk of toxicity," said Metin Kurtoglu, MD, PhD, Chief Medical Officer at Cartesian. "Descartes-08's enhanced safety features enable use for of a wide range of diseases, from multiple myeloma and other cancers to autoimmune diseases."

"Cartesian's fully integrated approach to R&D and a laser focus on internal cGMP manufacturing have enabled it to quickly translate three promising programs to clinical development," said John LaMattina, Pfizer's former President of Global R&D and a Senior Advisor to Cartesian. "I am impressed by the team's rapidly expanding pipeline of innovative cell and gene therapies. Development of a potent yet safer CAR-T allows for potential treatment of a wide range of autoimmune diseases. It also enables use of CAR-T earlier in the treatment paradigm for multiple myeloma and other cancers."

About Generalized Myasthenia Gravis

Generalized myasthenia gravis is a chronic auto-immune condition in which auto-antibodies attack specific proteins at the neuro-muscular junction. This disrupts the way that nerves can communicate with muscles, resulting in muscle weakness and fatigue. Both men and women are impacted equally, and it can occur at any age and in any race. Myasthenia gravis is a rare disease impacting almost 200,000 patients in the US, EU and Japan. Those living with GMG can experience a variety of symptoms, including drooping eyelids and double vision as well as severe muscular weakness that can result in life-threatening weakness of muscles of respiration.

About Cartesian Therapeutics

Founded in 2016, Cartesian is a fully-integrated, clinical-stage biopharmaceutical company developing potent yet safer cell and gene therapies designed to benefit the broadest range of patients with cancer and autoimmune diseases. Cartesian has three programs in clinical development, each under open INDs with the U.S. FDA. The lead investigational therapy, Descartes-08, is a CD8+ CAR T-cell therapy with a defined and predictable half-life, enabling repeat dosing to maximize potential for potency while minimizing risk of toxicity. Descartes-08 is currently in Phase I/II clinical trials to treat patients with multiple myeloma and generalized myasthenia gravis in an outpatient setting. All investigational therapies are manufactured at Cartesian's wholly-owned, state-of-the-art cGMP manufacturing facility in Gaithersburg, MD. Cartesian's commanding IP position benefits in part from a broad, exclusive patent license from the National Cancer Institute. For more information on Cartesian's clinical trials, visit www.cartesiantherapeutics.com.

About the University of Miami Miller School of Medicine

The University of Miami Leonard M. Miller School of Medicine is an academic medical center that transforms lives through teaching, research and service. We achieve transformation through innovative education programs that train the next generation of

medical leaders, groundbreaking biomedical research that has lifesaving global impact, and world-class compassionate clinical care that promotes better health throughout our community. The Neuromuscular Division in the Department of Neurology is home to the largest myasthenia gravis clinic in the region, with multiple specialists dedicated to providing world-class care for patients with this rare autoimmune disorder, and also providing opportunities for MG patients to participate in groundbreaking research.

Media Contacts:

Juliette Bogus for Cartesian Therapeutics
juliettebogus@presscommpr.com
410-980-5687

Kai T. Hill for University of Miami Health System Miller School of Medicine
khill@med.miami.edu
305-243-3249