Cartesian Therapeutics to Present Late-Breaking Data from Phase 1/2a Trial of RNA CAR T-cell Therapy in Patients with Generalized Myasthenia Gravis

May 9, 2022

– Data Presentation Today at 14th Myasthenia Gravis Foundation of America International Conference on Myasthenia and Related Disorders in Miami –

GAITHERSBURG, Md., May 10, 2022 – Cartesian Therapeutics, a fully integrated clinical-stage biotechnology company pioneering RNA cell therapy in and beyond oncology, will present late-breaking interim data from its Phase 1/2a clinical trial of Descartes-08 in patients with generalized Myasthenia Gravis (MG) today at the 14th Myasthenia Gravis Foundation of America (MGFA) International Conference on Myasthenia and Related Disorders. The conference is in Miami, May 10–12, 2022.

The oral presentation, "RNA CAR T-cell Therapy for Myasthenia Gravis," will be delivered today, Tuesday, May 10 at 10:50 a.m. ET by principal investigator Volkan Granit, M.D., of the University of Miami. A poster, "Phase 1b/2a Study of Autologous mRNA-Engineered Anti- B-cell Maturation Antigen Chimeric Antigen Receptor T-cells for Treatment of Severe Generalized Myasthenia Gravis," will be presented at the conference today and tomorrow.

"I am very encouraged by the interim analysis findings for this RNA CAR T-Cell therapy," said Dr. Granit. "The treatment appears safe and well tolerated, and the extent of clinical improvement seen in our early participants is remarkable. If future data in a controlled study corroborate these findings, Descartes-08 would become a welcome addition to our armamentarium for treating MG."

"We look forward to sharing these exciting interim data with the myasthenia community," said Miloš Miljković, M.D., Chief Medical Officer at Cartesian Therapeutics. "Descartes-08 therapy is intended to clear reservoirs of pathogenic long-lived plasma cells, a central component of many autoimmune diseases including MG. Consistent with this novel and broadly applicable mechanism of action, we plan to study Descartes-08 for other autoimmune diseases."

Descartes-08 Phase 1/2a Interim Data

The ongoing Phase 1/2a study (NCT 04146051) is split into two parts. The first part (dose escalation) assessed the safety and manufacturability of Descartes-08 administered in three ascending doses to each of three patients with severe, treatment-refractory generalized MG. Descartes-08 was well tolerated, with no cytokine release syndrome (CRS) or other serious product-related adverse events. All patients experienced at least a full class improvement in MGFA Clinical Classification and improvement on disease severity scales. Of note, mean improvement in the Myasthenia Gravis Composite (MGC) scale, which integrates both objective and subjective measures of disease severity, was over 50% three months after Descartes-08 administration. The second part (dose expansion) is intended to assess the safety and preliminary efficacy of Descartes-08 when administered repeatedly at a high dose on three different treatment schedules. To date, two patients enrolled into the weekly dosing cohort have received all six treatments and completed their Week 10 follow-up visit; both patients had significant disease (MGC >22) at baseline. Descartes-08 treatment was well tolerated. Both patients experienced dramatic and sustained improvements on all disease severity scales. Remarkably, at the 10 Week visit, the MGC score improved from 27 to 2 for the first patient and from 23 to 3 for the second patient.

About the Phase 1b/2a Clinical Trial

The ongoing Phase 1/2a trial (NCT04146051) is a multi-center open-label clinical study to determine the safety, manufacturing feasibility and preliminary efficacy of Descartes-08 in generalized MG. This study aims to enroll approximately 18 patients. For more information, visit cartformg.org.

About Descartes-08

Descartes-08 is an autologous BCMA-targeting RNA-modified CAR T-cell therapy. In early-stage clinical studies to date, Descartes-08 has been safe and well-tolerated in patients with MG and multiple myeloma.

About Cartesian Therapeutics

Founded in 2016 and with three assets in clinical trials, Cartesian is the leader in RNA cell therapy. With a mission to unleash the full potential and reach of cell therapy with RNA engineering, Cartesian is pioneering RNA cell therapies in and beyond oncology with products in development for autoimmune, oncologic and respiratory disorders. All investigational therapies are manufactured at Cartesian's wholly owned, state-of-the-art cGMP manufacturing facility in Gaithersburg, MD, enabling complete control of product quality, production schedules and costs while accelerating clinical translation of discoveries. For more information visit www.cartesiantherapeutics.com.

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