



Cartesian Therapeutics Receives FDA Rare Pediatric Disease Designation for Descartes-08 for the Treatment of Juvenile Dermatomyositis

September 9, 2024

IND filing for Phase 2 pediatric basket study on track for year-end

GAITHERSBURG, Md., Sept. 09, 2024 (GLOBE NEWSWIRE) -- Cartesian Therapeutics, Inc. (NASDAQ: RNAC) (the "Company"), a clinical-stage biotechnology company pioneering mRNA cell therapy for autoimmune diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to Descartes-08 for the treatment of juvenile dermatomyositis (JDM).

Descartes-08, Cartesian's lead mRNA cell therapy candidate, is an autologous mRNA-engineered chimeric antigen receptor T-cell therapy (mRNA CAR-T) product candidate targeting B-cell maturation antigen (BCMA). Descartes-08 is designed to be administered without preconditioning chemotherapy and does not use integrating vectors.

"We are pleased that the FDA recognizes the potential of Descartes-08 to serve as a meaningful therapeutic option for this underserved pediatric patient population," said Carsten Brunn, Ph.D., President and Chief Executive Officer of Cartesian. "Leveraging our novel mRNA platform, we are committed to our mission of expanding the reach of cell therapy to patients with autoimmune diseases. We remain on track to file an Investigational New Drug application for a Phase 2 pediatric basket study focused on neurology and rheumatology autoimmune indications, including JDM, by year-end."

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. Under the FDA's Rare Pediatric Disease Designation and Voucher Program, if Descartes-08 is approved for marketing in JDM, Cartesian may qualify for a priority review voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product.

About Juvenile Dermatomyositis

Juvenile Dermatomyositis (JDM) is a rare pediatric autoimmune disorder marked by pathognomonic skin rash and muscle inflammation affecting multiple organ systems including the joints, heart, lungs, kidneys, eyes, and gastrointestinal systems. The symptoms of JDM can range from mild to life-threatening and symptoms often include fatigue, joint pain, muscle weakness and fever. JDM impacts approximately 4,000 people in the United States.

About Cartesian Therapeutics

Cartesian Therapeutics is a clinical-stage company pioneering mRNA cell therapies for the treatment of autoimmune diseases. The Company's lead asset, Descartes-08, is an mRNA CAR-T in Phase 2b clinical development for patients with generalized myasthenia gravis and Phase 2 development for systemic lupus erythematosus, with a Phase 2 basket trial planned in additional autoimmune indications. The Company's clinical-stage pipeline also includes Descartes-15, a next-generation, autologous anti-BCMA mRNA CAR-T. For more information, please visit www.cartesiantherapeutics.com or follow the Company on [LinkedIn](#) or [X](#), formerly known as Twitter.

Forward Looking Statements

Any statements in this press release about the future expectations, plans and prospects of the Company, including without limitation, statements regarding the Company's plan to develop Descartes-08 in various pediatric indications, the ability of Descartes-08 to be administered in an outpatient setting or without the need for preconditioning lymphodepleting chemotherapy, the Company's in-house manufacturing capabilities, the potential of the Company's technology to enable precision control and optimization of engineered cells for diverse cell therapies leveraging multiple modalities, the potential of Descartes-08, or any of the Company's other product candidates to treat myasthenia gravis, systemic lupus erythematosus, multiple myeloma, or any other disease, the anticipated timing or the outcome of ongoing and planned clinical trials, studies and data readouts, the anticipated timing or the outcome of the FDA's review of the Company's regulatory filings, the Company's ability to conduct its clinical trials and preclinical studies, the timing or making of any regulatory filings, the anticipated timing or outcome of selection of developmental product candidates, the ability of the Company to consummate any expected agreements and licenses and to realize the anticipated benefits thereof, the novelty of treatment paradigms that the Company is able to develop, the potential of any therapies developed by the Company to fulfill unmet medical needs, and enrollment in the Company's clinical trials and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "hypothesize," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including, but not limited to, the following: the uncertainties inherent in the initiation, completion and cost of clinical trials including proof of concept trials, including uncertain outcomes, the availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a particular clinical trial will be predictive of the final results of that trial and whether results of early clinical trials or preclinical studies will be indicative of the results of later clinical trials, the ability to predict results of studies performed on human beings based on results of studies performed on non-human subjects, the unproven approach of the Company's technology, potential delays in enrollment of patients, undesirable side effects of the Company's product candidates, its reliance on third parties to conduct its clinical trials, the Company's inability to maintain its existing or future collaborations, licenses or contractual relationships, its inability to protect its proprietary technology and intellectual property, potential delays in regulatory approvals, the availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements, the Company's recurring losses from operations and negative cash flows, substantial fluctuation in the price of the Company's common stock, risks related to geopolitical conflicts and pandemics and other important factors discussed in the "Risk Factors" section of the Company's most recent Annual Report on Form 10-K and subsequently filed Quarterly Reports on Form 10-Q, and in other filings that the Company makes with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent the Company's views only as of the date of its publication and should not be relied upon as representing its views as of any subsequent date. The Company specifically disclaims any intention to update any forward-looking statements included in this press release, except as required by law.

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Source: Cartesian Therapeutics, Inc.