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Renovacor Announces Pipeline Expansion with New Research Program for Multiple Genetic Segments of Arrhythmogenic Cardiomyopathy

Research collaboration with the University of Utah's Nora Eccles Harrison Cardiovascular Research and Training Institute expands pipeline with the addition of an AAV gene therapy program for multiple genetic segments of arrhythmogenic cardiomyopathy

July 12, 2022 04:05 PM Eastern Daylight Time

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Renovacor, Inc. (NYSE: RCOR), a biotechnology company focused on delivering innovative precision therapies to improve the lives of patients and families battling genetically-driven cardiovascular and mechanistically-related diseases, today announced it has expanded its pipeline to advance an AAV gene therapy program as a potential precision therapy for three genetic segments of arrhythmogenic cardiomyopathy (ACM). To accelerate this new program, Renovacor has entered into a research collaboration with the University of Utah's Nora Eccles Harrison Cardiovascular Research and Training Institute (CVRTI). The terms of the research agreement grant Renovacor an option for an exclusive license to inventions generated from the collaboration.

The research collaboration will focus on a protein discovered by University of Utah scientists that has the potential to address multiple genetic segments of ACM. The new program is being developed as an AAV-based gene therapy to treat potentially life-threatening arrhythmias associated with the disease by restoring gap junction protein trafficking and gap junction communication between heart muscle cells. The program will be developed for the three largest genetic segments of ACM: plakophilin-2 (*PKP2*), desmoglein-2 (*DSG2*), and desmoplakin (*DSP*) associated ACM. Currently available treatment options do not address the trafficking defects central to each of the these genetically-driven forms of ACM.

The collaboration leverages positive proof-of-concept data generated in a genetic mouse model of ACM that was performed by the Shaw Lab, led by Robin Shaw, M.D., Ph.D., Professor of Medicine at the University of Utah and Director of the CVRTI. These data demonstrate restoration of gap junction trafficking to the intercalated disc and a significant reduction in premature ventricular contractions (PVCs). PVCs are a hallmark of ACM and key drivers of potentially lethal ventricular arrhythmias.

"Renovacor's pipeline expansion with this new AAV gene therapy research program for multiple genetic segments of ACM further demonstrates our precision medicine approach to develop potentially transformative therapies that target core biological drivers of serious cardiovascular diseases," said Matt Killeen, Ph.D., Chief Scientific Officer of Renovacor. "We believe we have found the ideal program and partner to leverage our expertise in heart muscle biology to discover and develop a novel gene therapy that could one day address a significant unmet medical need."

“We are thrilled to have Renovacor as a partner to continue the research into these very important genetic drivers of ACM,” said Robin Shaw, M.D., Ph.D., Director of the CVRTI. “ACM is a serious disease of heart muscle that can lead to life-threatening, intractable arrhythmias. The team at Renovacor are experts in the understanding the importance of heart muscle biology, which makes them the ideal development partner to advance a novel, precision medicine approach for ACM. By seeking to understand and address a key causal disease pathway in ACM, together we hope to develop a therapeutic that could help improve the lives of patients who are living with this serious form of cardiomyopathy.”

Arrhythmogenic cardiomyopathy (ACM) is a heritable heart muscle disorder that can affect the left and right ventricle. It is characterized by a heightened risk of potentially lethal ventricular arrhythmias, fibrofatty replacement of myocardial tissue, and in some patients, heart failure.[1,2] It is recognized as a disease of the desmosome, with well-defined genetic drivers. The prevalence of ACM is estimated to range from 1 case in 1,000 persons to 1 case in 5,000, with an average age of diagnosis of approximately 30 years.[1-3] Current treatment options aim to prevent potentially life-threatening arrhythmias and progression to end-stage disease, but they do not target the underlying genetics or disease biology and, as such, patients can continue to experience serious breakthrough events.[1-2]

About Renovacor

Renovacor is a biotechnology company focused on delivering innovative precision therapies to improve the lives of patients and families battling genetically-driven cardiovascular and mechanistically-related diseases. The company’s lead program in BAG3-associated dilated cardiomyopathy (DCM) uses gene transfer technology to address the monogenic cause of this severe form of heart failure. Renovacor’s vision is to bring life-changing therapies to patients living with serious genetic cardiovascular and related diseases, by developing medicines that target the underlying cause of disease and provide a transformative benefit and significant improvement to quality of life.

About the University of Utah

The University of Utah is the state’s flagship institution of higher education, with 18 schools and colleges, more than 100 undergraduate and 90 graduate degree programs, and an enrollment of more than 32,000 students. The University serves as a catalyst for the regional innovation economy, having supported the launch and growth of over 300 companies and conducted more than $640 million in annual research.

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1. Austin KM et al *Nat Rev Cardiol*. 2019 Sep; 16(9): 519–537
2. Corrado D, et. al, *N Engl J Med* 2017;376:61-72
3. McNally E (2017) in: Adam MP, Mirzaa GM, Pagon RA, GeneReviews®

Forward-Looking Statements

This press release contains certain forward-looking statements within the meaning of the “safe harbor” provisions of the United States Private Securities Litigation Reform Act of 1995, as amended, including statements regarding the anticipated development of Renovacor’s product candidates and development programs. These forward-looking statements generally are identified by the words “believe,” “project,” “expect,” “anticipate,” “estimate,” “intend,” “strategy,” “future,” “opportunity,” “plan,” “may,” “should,” “will,” “would,” “will be,” “will continue,” “will likely result,” and similar expressions. These forward-looking statements are based upon current estimates and assumptions of the Company and its management and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release. Factors that may cause actual results to differ materially from current expectations include, but are not limited to: competition, the ability of the company to grow and manage growth, maintain relationships with customers and suppliers and retain its management and key employees; the Company's ability to successfully advance its current and future product candidates through development activities, preclinical studies and clinical trials and costs related thereto; the timing, scope and likelihood of regulatory filings and approvals, including final regulatory approval of our product candidates; changes in applicable laws or regulations; the possibility that the Company may be adversely affected by other economic, business or competitive factors; the Company’s estimates of expenses and profitability; the evolution of the markets in which the Company

competes; the ability of the Company to implement its strategic initiatives and continue to innovate its existing products; the ability of the Company to defend its intellectual property; the impact of the COVID-19 pandemic on the Company's business, supply chain and labor force; and the risks and uncertainties described in the "Risk Factors" section of the Company's annual and quarterly and reports filed the Securities Exchange Commission. These filings identify and address important risks and uncertainties that could cause actual events and results to differ materially from those contained in the forward-looking statements. Forward-looking statements speak only as of the date they are made. Readers are cautioned not to put undue reliance on forward-looking statements, and Renovacor assumes no obligation and does not intend to update or revise these forward-looking statements, whether as a result of new information, future events, or otherwise. Renovacor gives no assurance that it will achieve its expectations.

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