Press Release

AMO Pharma Enters into License Agreement with Population Health Research Institute (PHRI) and Venca Research Inc. to Advance Largest-Ever Study in Treatment of Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC)

Phase 2 proof-of-concept trial with AMO Pharma investigative therapy AMO-02 now underway at 17 sites across Canada; 120 patients expected to enroll

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LONDON, Sept. 8, 2025 /PRNewswire/ -- AMO Pharma Limited ("AMO Pharma"), a privately held clinical-stage specialty biopharmaceutical company focusing on rare genetic disorders with limited or no treatment options, today announced a license agreement with Population Health Research Institute (PHRI), a joint global health research institute of McMaster University and Hamilton Health Sciences, and Venca Research Inc. The agreement will support the development and potential manufacturing, and commercialization of AMO-02 in arrhythmogenic right ventricular cardiomyopathy (ARVC). Recruitment is now underway with the first patient randomized in the PHRI TaRGET Phase 2 proof-of-concept clinical trial evaluating AMO-02 for the treatment of ARVC. The study will be the largest clinical trial of a therapeutic ever conducted in this rare, heritable cardiomyopathy.

"AMO Pharma is committed to advancing and supporting research that can lead to new first-in-class disease-modifying therapies for serious, life-threatening conditions such as ARVC," said Mike Snape, Chief Scientific Officer at AMO Pharma. "The **recent publication** in JACC: Basic to Translational Science reinforces the growing body of evidence supporting glycogen synthase kinase-3 beta (GSK3β) inhibition as a potential treatment strategy for ARVC and the broader ACM spectrum, which is central to our approach with AMO-02. We look forward to partnering with PHRI to advance this landmark precision cardiology program, which has the potential to change the treatment landscape for patients living with ARVC by targeting the underlying disease biology. This collaboration builds on extensive prior research highlighting AMO-02's mechanism of action indicating the potential to correct abnormal GSK3β activity in the heart, and its established unique profile as a safe and well-tolerated GSK3β inhibitor."

ARVC is a rare, inherited cardiomyopathy that can lead to heart failure, malignant ventricular arrhythmias, and sudden cardiac death. It is most often caused by genetic mutations in desmosomal genes that trigger abnormal activation of the enzyme GSK3β in cardiac cells. ARVC is a subtype of arrhythmogenic cardiomyopathy (ACM), a broader condition that can also affect the left ventricle or both ventricles.

"In preclinical studies, insertion of human ARVC gene mutations into mice reproduced all the major signs and symptoms of the disease," said Dr. Jason Roberts, principal investigator of the study and PHRI scientist. "When studied in these models, AMO-02 reduced arrhythmias and cardiomyopathy, and both prevented progression and reversed existing disease features. These findings strongly underscore its potential to address the underlying drivers of ARVC and potentially offer a much-needed therapeutic option for patients."

The TaRGET study is a randomized, double-blind, placebo-controlled trial evaluating AMO-02 in patients with genotype-positive ARVC. The primary endpoint is the change from baseline in mean premature ventricular contractions per 24 hours, measured by seven-day Holter monitoring compared to placebo at six months of treatment. Secondary endpoints include right ventricular strain on echocardiography, frequency of implantable cardioverter-defibrillator therapies, and episodes of sustained ventricular tachycardia. Under the terms of the agreement, AMO Pharma will work closely with PHRI on regulatory interactions and study oversight. First data from the trial are expected in the second quarter of 2027.

About AMO Pharma
AMO Pharma is a clinical-stage specialty biopharmaceutical company working to identify and advance promising therapies for the treatment of serious and debilitating diseases in patient populations with significant areas of unmet need, including rare and severe childhood onset neurogenetic disorders with limited or no treatment options. In addition to developing AMO-02 for DM1, the company is also progressing AMO-01 as a clinical stage treatment for Phelan-McDermid syndrome and AMO-04 as a clinic-ready potential medicine for Rett syndrome and related disorders. AMO-02, AMO-01 and AMO-04 are investigational medicines that have not yet been approved for the treatment of patients anywhere in the world. Advice provided to AMO Pharma by regulators is under the condition that any scientific advice given is not legally binding with regards to any future application for the product concerned. Furthermore, advice cannot be taken as indicative of any future agreed position.

For more information, please visit the AMO Pharma website at http://www.amo-pharma.com/.

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