



## Press Release

# AMO Pharma in Collaboration with Population Health Research Institute (PHRI) and Venca Research Inc. Announces FDA Advice on Potential Phase 3 Development Path for AMO-02 in Treatment of Arrhythmogenic Cardiomyopathy (ACM)

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*FDA feedback provides clarity on trial protocol and primary endpoint that could support a potential Phase 3 study of the investigational product AMO-02 for the treatment of ACM.*

LONDON, Oct. 7, 2026 /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, in collaboration with Population Health Research Institute (PHRI) and Venca Research Inc., today announced that the U.S. Food and Drug Administration (FDA) has provided advice via a pre-IND consultation on the design of a potential Phase 3 trial evaluating AMO-02 in arrhythmogenic cardiomyopathy (ACM), including comments on the primary outcome measure.

"This feedback represents an important step forward in defining the development pathway for AMO-02 in ACM," said Dr. Mike Snape, chief executive officer of AMO Pharma. "ACM is a serious and potentially life-threatening disease with limited treatment options. The FDA's feedback provides greater clarity on the evidence that would be needed and will help inform our planning as we continue the ongoing Phase 2 TaRGET study."

ACM 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 GSK3 $\beta$  in cardiac cells. Arrhythmogenic right ventricular cardiomyopathy (ARVC) is the most common subtype of arrhythmogenic cardiomyopathy (ACM) and primarily affects the right ventricle.

The FDA addressed the definition of ACM for a proposed indication and provided the following advice on a potential efficacy endpoint: "In principle, an endpoint that captures effects on ICD therapies for VT such as shock or anti-tachycardia pacing and sustained

symptomatic VT events could be used to demonstrate the effectiveness of your product for the treatment of ACM."

"People living with ACM face the ongoing risk of life-threatening arrhythmias and sudden cardiac death, yet treatment options remain largely focused on managing symptoms and reducing immediate risks rather than targeting the underlying disease," said Dr. Jason Roberts, principal investigator of the **TaARGET** study and a scientist at PHRI, a joint institute of McMaster University and Hamilton Health Sciences. "The FDA's advice brings us closer to a clear clinical development path for evaluating the potential of AMO-02. We look forward to continuing to build the evidence needed to advance this important research."

The ongoing PHRI-led Phase 2 TaARGET study is a randomized, double-blind, placebo-controlled trial evaluating AMO-02 in patients with genotype-positive ACM with a planned recruitment of 120 patients at 17 sites in Canada (ClinicalTrials.gov ID: NCT06174220). The primary endpoint is the change from baseline in mean premature ventricular contractions, measured by seven-day Holter monitoring compared to placebo at six months of treatment. Secondary endpoints include ventricular strain on echocardiography, frequency of implantable cardioverter-defibrillator therapies, and episodes of sustained ventricular tachycardia. TaARGET is a Phase 2 proof-of-concept study; its primary endpoint differs from the endpoint discussed with FDA for a potential Phase 3 study. Under the terms of the agreement, AMO Pharma has been working closely with PHRI on regulatory interactions and study oversight. First data from the trial are expected in 2028.

### **About AMO Pharma**

AMO Pharma is a clinical-stage specialty biopharmaceutical company working to identify and advance investigational 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-02 in clinical trials for the treatment of arrhythmogenic cardiomyopathy (ACM). Advice provided to AMO Pharma by regulators is under the condition that any scientific advice given is not legally binding with regard to any future application for the product concerned. FDA feedback is non-binding and does not ensure agreement on any future study design or acceptance of any future marketing application. This press release contains forward-looking statements regarding anticipated Phase 3 trial initiation and development plans. Such statements are subject to risks and uncertainties, including regulatory, clinical, and operational factors, that could cause actual results to differ materially.

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

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