XyloCor Therapeutics Commences Phase 2 Component of Phase 1/2 EXACT Clinical Study of XC001 Gene Therapy for Refractory Angina

- *Independent Data Monitoring Committee authorized proceeding to Phase 2 at highest dose level tested following review of clinical safety data from the Phase 1 dose escalation*
- *Phase 2 clinical data readouts on safety and efficacy of XC001 anticipated in 2022*
- *Company plans to commence study startup of XC001 as an adjunct to CABG in 2H21, and clinical studies in additional cardiovascular indications are under discussion*
- *Progress highlights potential of gene therapy in cardiovascular disease*

Wayne, PA, July 27, 2021 – XyloCor Therapeutics, a clinical-stage biopharmaceutical company developing novel gene therapies for cardiovascular disease, today announced the completion of the Phase 1 dose-escalation component of its Phase 1/2 clinical trial (EXACT) of XC001 (encoberminogene rezmadenovec), its lead investigational gene therapy candidate for patients with refractory angina who have no further treatment options.

Following a review of clinical data from the Phase 1 dose escalation component of the study, the Independent Data Monitoring Committee (IDMC) authorized proceeding to the Phase 2 component of the study at the highest dose tested. Since the IDMC authorization, three patients have been dosed in the Phase 2 expansion cohort. This progress highlights the potential for gene therapy to go beyond rare diseases and to address larger patient populations with significant unmet needs, such as chronic cardiovascular diseases.

XyloCor also confirms that it plans to submit an additional Phase 2 clinical study to the U.S. Food and Drug Administration (FDA) for XC001 as adjunctive therapy to coronary artery bypass grafting (CABG) in 2H21. The company also plans other clinical studies in additional cardiovascular indications, including heart failure caused by ischemic heart disease and as adjunctive therapy to percutaneous coronary intervention.

“Patients with refractory angina are forced to live with the ongoing burden of a disease that limits their activities on a daily basis due to chest pain,” said Thomas Povsic, M.D., Ph.D., Duke University cardiologist and National Principal Investigator for the EXACT study. “With a unique mechanism of action that restores blood flow to the heart via the creation of new blood vessels, XC001 represents a novel therapeutic approach for patients who have exhausted other medical and surgical options. It is very exciting to now move forward with exploring XC001’s potential in the Phase 2 portion of EXACT as a one-time therapy for patients with refractory angina.”

“In our mission to deliver safe and effective gene therapies that transform the lives of people with cardiovascular disease, we are excited to achieve this important milestone and advance into the Phase 2 portion of our study,” said Al Gianchetti, President and Chief Executive Officer of XyloCor Therapeutics. “XC001 has enormous potential to significantly improve the lives of patients with refractory angina. We are grateful for the support of patients and their families, as well as the EXACT trial investigators as we continue to study the safety and efficacy of XC001 and look forward to reporting results in 2022.”

XC001 (encoberminogene rezmadenovec) is a novel, investigational gene therapy designed to stimulate the growth of new blood vessels in the heart, in order to bypass diseased vessels and improve coronary blood flow. XC001 delivers the gene for vascular endothelial growth factor (VEGF), a naturally occurring protein, in targeted myocardial cells, thus stimulating the creation of new blood vessels via a process called angiogenesis. XC001 employs a proprietary multi-isoform VEGF expression cassette that has been optimized to maximize expression of VEGF. XC001 has been granted Fast Track designation by the FDA for study in refractory angina. XyloCor commenced the EXACT Trial, a Phase 1/2 study of XC001 in chronic refractory angina, in 2020.

About the EXACT Study

The Epicardial Delivery of XC001 Gene Therapy for Refractory Angina Coronary Treatment (EXACT) clinical trial is a Phase 1/2 multicenter, open-label, single-arm, dose-escalation trial. 12 subjects (n=3 per dose cohort) who have refractory angina were enrolled into four ascending dose groups, to be followed by an expansion phase of the trial with 21 additional subjects at the highest tolerated dose. The trial is designed to assess the preliminary safety and efficacy of XC001. The investigational gene therapy is administered directly to the heart muscle through a mini-thoracotomy by an experienced cardiac surgeon. The EXACT Trial is being conducted at top cardiovascular research sites across the United States.

About Chronic Refractory Angina

In the United States, coronary artery disease is a leading cause of death and disability. Chronic angina pectoris occurs when the heart muscle does not receive sufficient oxygen resulting in chest pain. This is usually due to atherosclerotic plaques that block the coronary arteries. Refractory angina is a growing problem that occurs in patients with chronic angina who are symptomatic despite optimal medical therapy and are no longer eligible for mechanical interventions like percutaneous coronary intervention (PCI) and coronary artery bypass grafting (CABG). These patients currently have no treatment options and are frequently highly symptomatic, which severely impacts their quality of life, and may exacerbate comorbidities and cause further deterioration of their health status. Refractory angina results in significant consumption of healthcare resources, including visits to the emergency department as a result of patients' chest pain. An estimated one million people suffer from refractory angina in the United States.

About XyloCor

XyloCor Therapeutics is a private, clinical-stage biopharmaceutical company developing potential best-in-class gene therapies to transform outcomes for patients with cardiovascular disease. The Company's lead product candidate, XC001, is in clinical development to investigate use for patients with refractory angina for which there are no treatment options. XyloCor has a second preclinical investigational product, XC002, in discovery stage, being developed for the treatment of patients with cardiac tissue damage from heart attacks. The company, which was co-founded by Ronald Crystal, MD, and Todd Rosengart, MD, has an exclusive license from Cornell University. For more information, visit www.xylocor.com.

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