Gene therapy treatment increasing body's signal for new blood vessel growth shows promise

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Final 12-month data from the EXACT trial demonstrates safety and efficacy results for a vascular endothelial growth factor (VEGF) gene therapy treatment for patients who have advanced coronary artery disease (CAD). The late-breaking results were presented today at the Society for Cardiovascular Angiography & Interventions (SCAI) 2024 Scientific Sessions.

CAD, also known as coronary heart disease or ischemic heart disease, affects about 20.5 million U.S. adults—making it the most common type of heart disease in the United States. Often, the first sign of CAD is a heart attack, triggered by a rupture of plaque accumulated in the arteries supplying blood to the heart.

Over time, plaque narrows these arteries, blood flow diminishes, leading to angina—a condition characterized by chest pain due to insufficient oxygen-rich blood supply to the heart muscle. In patients with the most severe form, angina can be disabling, and additional medications, procedures or surgery may not be effective. There is a need for therapies for such a serious condition.

The EXACT trial assesses the safety and preliminary efficacy of the gene therapy XC001 in patients with "no option" refractory angina (NORA). The gene vector is designed to more effectively and safely increase the body's own signal for new blood vessel growth. Effectiveness was measured primarily by exercise capacity, degree of impairment of blood flow to the heart, and angina frequency and severity.

Among the 32 patients with NORA, the gene therapy XC001 appeared safe with no serious adverse effects due to the drug. Surgical delivery was generally well-tolerated. Early benefits of XC001 are promising in
relation to improvements in exercise duration, decreased symptoms, and improved blood flow in patients' hearts.

Total exercise duration increased from a mean of 359.9 seconds at baseline to 448.2 at three months, 449.2 at six months, and 477.6 at 12 months. Total myocardial perfusion deficit on positron emission tomography imaging decreased by 10.2% at three months, 14.3% at six months, and 10.2% at 12 months—demonstrating a reduction in impaired blood flow.

The time to onset of ST depression during exercise tolerance testing increased by 105.2 at three months, 113.6 at six months, and 103.1 seconds at 12 months. Angina frequency decreased by -7.7 at three months, -6.6 at six months, and -8.8 episodes at 12 months. Angina class improved in 81% of participants at six months.

"Findings from the EXACT trial may not impact clinical practice immediately, but it will lay the groundwork for future studies in this patient population," said Thomas J. Povsic, MD, Interventional Cardiologist at Duke University Medical Center, and Kenta Nakamura, MD, Interventional Cardiologist at the University of Washington Medical Center, and lead authors of the study.

"We hope this will lead to the development of a new treatment for patients with coronary disease or as a frontline therapy for angina, especially for those who are not candidates for additional conventional therapies like PCI or CABG."

**More information:** "VEGF Gene Therapy Improves Exercise Time, Ischemia, and Symptoms in Patients with Refractory Angina: Results of the Phase II EXACT Trial," Thursday, May 2, 2024; 9:31-9:38 AM PT,
Provided by Society for Cardiovascular Angiography and Interventions

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