



Editas Medicine Receives Approval to Initiate First-In-Human Clinical Trial of EDIT-401 in Patients with Hyperlipidemia

October 8, 2026

Phase 1/2 clinical trial of EDIT-401 cleared to initiate dosing in patients who require additional LDL-C lowering

Company remains on track to begin patient dosing this year and report initial safety and tolerability data in the first quarter of 2027

New EDIT-401 preclinical data demonstrating durability of LDL-C reduction to be presented in oral presentation at AHA Scientific Sessions 2026

CAMBRIDGE, Mass., Oct. 08, 2026 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company focused on developing transformative medicines for serious diseases, today announced it has received approval to initiate the Phase 1/2 Strive™ clinical trial of EDIT-401 in patients with Heterozygous Familial Hypercholesterolemia (HeFH) in Australia. The trial has received Human Research Ethics Committee (HREC) approval and completed the Clinical Trial Notification (CTN) process with Australia's Therapeutic Goods Administration (TGA) and has also been submitted for review in New Zealand. If approved in New Zealand, patients in both countries will be eligible to enroll in the Strive trial. The Company also announced that new preclinical data, demonstrating durability of LDL-C reduction with EDIT-401, will be released via an oral presentation at the American Heart Association (AHA) Scientific Sessions 2026, taking place November 6-9, 2026, in Chicago, Illinois.

EDIT-401 is an experimental, potentially best-in-class *in vivo* gene editing medicine for the treatment of hyperlipidemia. Utilizing Editas' differentiated upregulation approach, EDIT-401 is designed to directly edit the LDLR gene to increase LDLR protein expression and reduce LDL-cholesterol (LDL-C) levels. In preclinical studies, EDIT-401 demonstrated ~90 percent or greater mean reduction in multiple atherogenic lipoproteins, including LDL-C, Lp(a), and ApoB in non-human primates. New preclinical data to be presented at AHA also demonstrated an LDL-C reduction of ~90 percent was maintained over 10 months in an ongoing study in non-human primates.

"The receipt of regulatory and ethics approval for clinical trial initiation in Australia is a significant step in advancing the clinical development of EDIT-401, a potentially transformative *in vivo* gene editing medicine designed to upregulate LDLR expression and reduce LDL-C levels in patients living with hyperlipidemia. We look forward to initiating the Strive study and generating our first-in-human data as we advance EDIT-401 through key clinical milestones in 2027," said Dan Ory, M.D., Chief Medical Officer, Editas Medicine. "We are also encouraged by the new EDIT-401 preclinical data to be presented at AHA, which further support its potential to provide durable, lifelong benefits to patients."

The Strive trial is designed to evaluate the safety, tolerability, and efficacy of a single dose of EDIT-401. The trial is designed in two parts: Part 1 is a single ascending dose, dose-finding, open-label trial. Part 2 will be a single-dose randomized, placebo-controlled expansion study. Editas has selected five clinical trial sites across Australia and New Zealand.

Editas expects to report initial safety and tolerability data in the first quarter of 2027. The Company plans to complete enrollment in Part 1, the dose-finding portion of the Phase 1/2 trial of EDIT-401, with topline safety and efficacy data results available later in 2027.

American Heart Association (AHA) Scientific Sessions 2026 Oral Presentation

Editas will present new preclinical data demonstrating the durability of EDIT-401, supporting its potential as a transformative therapy for people living with hyperlipidemia, at the AHA Scientific Sessions on Sunday, November 8.

Presentation Details:

Title: EDIT-401: *In Vivo* Gene Editing to Enhance LDLR Function for Durable LDL-C Reduction

Session Date and Time: Sunday, November 8, 2026, 3:30 - 4:45 p.m. CT

Session Title: Translational Gene and Epigenome Therapies For Lipid Management: A New Frontier

Presenter: Dan Ory, M.D., Chief Medical Officer, Editas Medicine

The final presentation can be accessed on the [AHA website](#). A copy of the presentation will also be posted to the "[Posters & Presentations](#)" section of the Company's website during the conference.

About EDIT-401

EDIT-401 is an experimental, potentially best-in-class *in vivo* gene editing medicine, based on Editas' differentiated upregulation approach. EDIT-401 is designed to treat hyperlipidemia by directly editing the LDLR gene to increase LDLR protein expression and reduce LDL-C levels. This targeted approach has demonstrated a favorable preclinical profile in both efficacy data and tolerability and supports the potential of EDIT-401 to deliver meaningful clinical outcomes for patients underserved by current lipid-lowering therapies.

About Heterozygous Familial Hypercholesterolemia (HeFH)

Heterozygous Familial Hypercholesterolemia (HeFH) is an inherited genetic disorder that leads to significantly elevated LDL-C levels from an early age. Individuals with HeFH are at high risk of heart disease, heart attack, or stroke if the condition is not identified and treated early. An estimated 1.2 million people in the United States are living with HeFH, though many remain undiagnosed. Elevated LDL-C, also known as hyperlipidemia, is a highly prevalent disease affecting over 70 million patients in the United States alone. Substantial unmet need exists across multiple at-risk segments of patients with hyperlipidemia, including the HeFH population.

About Editas Medicine

As a pioneering gene editing company, Editas Medicine is focused on translating the power and potential of CRISPR genome editing systems into a robust pipeline of transformative *in vivo* medicines for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize durable, precision *in vivo* gene editing medicines for a broad class of diseases. Editas Medicine is the exclusive

licensee of Broad Institute's Cas12a patent estate and Broad Institute and Harvard University's Cas9 patent estates for human medicines. For the latest information and scientific presentations, please visit www.editasmedicine.com.

Forward-Looking Statements

This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "should," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include statements regarding the initiation, timing, progress and results of the Company's planned clinical trials, including the Company's expectation to begin patient dosing in 2026 and complete enrollment in Part 1, the dose-finding portion of the Phase 1/2 trial of EDIT-401, with topline safety and efficacy data results available later in 2027; the timing for the Company's receipt and presentation of data from its preclinical and planned clinical studies, including reporting initial safety and tolerability data in the clinical trial of EDIT-401 in the first quarter of 2027; and the potential of, and expectations for, EDIT-401. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation, timing, progress, and results of preclinical studies and clinical trials; uncertainty regarding availability and timing of results from preclinical studies and clinical trials; uncertainties relating to planned regulatory submissions to initiate clinical trials, including that results of preclinical studies will warrant such submissions or that regulatory agencies may require additional preclinical studies, that regulatory submissions shall occur on the expected timelines and that regulatory authorities will provide clearance for trials to be initiated on the expected timelines or at all; and uncertainties as to whether the Company's cash resources are sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements for the period anticipated. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by the Company's subsequent filings with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release represent the Company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.

Investor and Media Contacts:

ir@editasmed.com

media@editasmed.com

