



Editas Medicine Reports New Preclinical Data Demonstrating Progress of EDIT-401 as Potential Treatment for Hyperlipidemia at the American Society of Gene and Cell Therapy 2026 Annual Meeting

May 14, 2026

New data demonstrate promising preclinical safety profile and durable LDL-C lowering of $\geq 90\%$ with single dose of EDIT-401 in non-human primates through ~6 months

CAMBRIDGE, Mass., May 14, 2026 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company developing transformative medicines for serious diseases, shared new preclinical data supporting the continued advancement of Editas' lead *in vivo* development candidate, EDIT-401, and its potential as a one-time treatment for hyperlipidemia, as well as the broader potential of the Company's differentiated upregulation strategy. The data is being presented this week at the 2026 Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) in Boston, including one oral presentation and two poster presentations, as well as one oral presentation at TIDES USA 2026: Oligonucleotide and Peptide Therapeutics Conference.

Key EDIT-401 data presented include:

- In an oral presentation at ASGCT, Editas reported that a single dose of EDIT-401 achieved ≥ 90 percent mean LDL-C reduction across all dose groups in non-human primates (NHPs).
 - ≥ 90 percent mean LDL-C reduction was achieved with only moderate levels (10-40 percent) of functional editing of LDLR alleles and ≥ 6 -fold mean increase in hepatic LDLR protein.
 - LDL-C lowering was rapid and remained durable across evaluated dose levels (1.5 mg/kg-3.0 mg/kg) through ~6 months.
 - Promising preclinical safety profile with no adverse clinical observations at therapeutically relevant dose (1.5 mg/kg).
 - The highest delivery of EDIT-401 was observed in the hepatocytes as compared to other non-target tissues with undetectable oocyte delivery.
- In an oral presentation at TIDES, Editas presented data demonstrating EDIT-401 dose-dependent LDL-C reduction in NHPs.
- In a poster presentation at ASGCT, Editas reported that data evaluating pharmacokinetics and pharmacodynamics of a single dose of EDIT-401(mu) across multiple dose levels in heterozygous *Ldlr* loss-of-function mice and wildtype mice support that dose adjustments may not be needed to achieve LDL-C lowering in Heterozygous Familial Hypercholesterolemia (HeFH) patients.

Additional *in vivo* upregulation findings from a poster presentation at ASGCT include:

- Data support leveraging DNA large language prediction models (DNA-LLM) to accelerate and streamline the pursuit of gene editing-based strategies designed to mitigate disease through augmentation of alternate or compensatory pathways and further highlight the broader potential of Editas' *in vivo* gene upregulation platform.

"These new EDIT-401 preclinical data, including durability of LDL-C reduction across a range of doses through ~6 months demonstrated in NHPs, strengthen our confidence in EDIT-401 as a potential one-time treatment to deliver meaningful and durable LDL-C lowering and support its continued advancement toward first-in-human clinical development," said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. "Further, the data presented also highlight the broader potential and differentiation of our upregulation strategy to generate new medicines across multiple disease areas."

The presentation details are listed below. Abstracts can be accessed on the ASGCT website, and the presentations will be posted on the [Editas Medicine website](#) during the conferences.

American Society of Gene and Cell Therapy (ASGCT) 2026 Annual Meeting, May 11-15

Oral Presentation:

Title: Preclinical Development of EDIT-401, a Durable *In Vivo* CRISPR Gene Editing Therapy That Upregulates LDLR Protein to Lower LDL-C

Session Date and Time: Thursday, May 14, 3:30 p.m. – 5:00 p.m. EDT

Session Title: Gene Therapy for Cardiovascular Diseases

Presentation Room: 206AB

Final Abstract Number: 380

Poster Presentations:

Title: Pharmacokinetics and Pharmacodynamics of *In Vivo* Gene Editing Therapy for Lowering LDL-C in Mice

Session Date and Time: Thursday, May 14, 5:00 p.m. – 6:30 p.m. EDT

Session Title: Thursday Poster Reception

Presentation Room: Exhibit and Poster Hall

Final Abstract Number: 3423

Title: *In Vivo* CRISPR-based Disruption of an Important Gene Repressor Element Upregulates a Compensatory Protein to Normalize Disease-Associated Biomarkers in a Knockout Mouse Disease Model

Session Date and Time: Wednesday, May 13, 5:00 p.m. – 6:30 p.m. EDT

Session Title: Wednesday Poster Reception

Presentation Room: Exhibit and Poster Hall

Final Abstract Number: 2324

TIDES USA 2026: Oligonucleotide and Peptide Therapeutics Conference, May 11-14

Oral Presentation:

Title: Transformative LDL Cholesterol Lowering *In Vivo* CRISPR Gene Editing Approach for Hyperlipidemia and Atherosclerotic Cardiovascular Disease

Session Date and Time: Wednesday, May 13, 8:30 a.m. – 9:00 a.m. EDT

Session Title: mRNA & Genome Editing: Technology & Applications

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 progress and results of the Company's preclinical studies and planned clinical trials, including the Company's expectation to initiate a first-in-human clinical trial of EDIT-401; 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; and that the Company will not be able to raise funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements. 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.

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Source: Editas Medicine, Inc.