



## Editas Medicine Nominates EDIT-401, an LDLR-Targeted Medicine, as Lead In Vivo Development Candidate

September 2, 2025

*EDIT-401 achieved ~90% mean LDL-C reduction with single dose in non-human primates*

*EDIT-401 on track for human proof-of-concept data by end of 2026*

*Strong cash position with operational runway into second quarter of 2027*

*Company-sponsored webinar on EDIT-401 today at 8:00 a.m. ET*

CAMBRIDGE, Mass., Sept. 02, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company, today announced the nomination of its lead *in vivo* development candidate, EDIT-401, an experimental, potential best-in-class, one-time therapy designed to significantly reduce LDL cholesterol (LDL-C) levels.

"Today's nomination of EDIT-401 as our lead *in vivo* development candidate is a significant milestone in our long-term vision to lead the field of *in vivo* programmable gene editing," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer of Editas Medicine. "At Editas Medicine, we believe *in vivo* is the future of medicine. With EDIT-401, we're advancing our differentiated upregulation strategy to develop a next-generation medicine that can deliver lasting impact for patients."

### About EDIT-401

EDIT-401 is an experimental, potentially transformative *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.

Key highlights of EDIT-401 include:

- Robust efficacy data with ~90 percent mean reduction of LDL-C in preclinical non-human primate studies, while standard of care therapies demonstrate a 40-60% mean reduction of LDL-C
- Potential one-time treatment designed for lifelong benefit
- Sizeable market potential with favorable health care system economics
- Attractive business model expected to align with typical biopharma margins
- Compelling preclinical data supporting rapid progression to human proof-of-concept data by end of 2026

### About Hyperlipidemia and Atherosclerotic Cardiovascular Disease (ASCVD)

Atherosclerotic cardiovascular disease (ASCVD) is the leading cause of death worldwide and imposes a significant burden on the US healthcare system with national expenditures projected to reach over \$300 billion in 2035. Elevated LDL-C is a major causal factor in ASCVD disease. 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.

"EDIT-401 leverages our *in vivo* gene editing platform to precisely target noncoding regions that regulate LDLR gene expression," said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer of Editas Medicine. "This targeted editing approach enables direct upregulation of LDLR, with the potential for EDIT-401 to deliver meaningful benefits beyond the current standard of care across multiple segments of the hyperlipidemia population."

### Hematopoietic Stem Cells Program Update

Editas remains committed to progressing its other programs and pipeline. The Company intends to continue work on optimizing candidates for its Hematopoietic Stem Cell (HSC) program but plans to focus its resources on the advancement of its lead EDIT-401 program to human proof-of-concept.

### Key Upcoming Planned Milestones

The Company expects to identify and disclose an additional target cell type or tissue by the end of 2025. By mid-2026, it plans to submit an investigational new drug (IND) or clinical trial application (CTA) for EDIT-401, with the goal of achieving *in vivo* human proof-of-concept data for EDIT-401 by the end of 2026.

### Cash Runway

As of June 30, 2025, the Company had cash, cash equivalents, and marketable securities of \$178.5 million. Editas re-confirmed its cash runway guidance and expects that its existing cash, cash equivalents, and marketable securities and the retained portions of the payments payable under its license agreement with Vertex Pharmaceuticals will enable the Company to fund its operating expenses and capital expenditure requirements into the

second quarter of 2027.

#### **Webinar Presentation Details:**

The Company will host a webinar on Tuesday, September, 2, 2025 at 8:00 a.m. ET to discuss the selection of EDIT-401 as its lead *in vivo* development candidate. The live and archived webcast of the Company's webinar presentation will be accessible through this [webcast link](#), or through the Events & Presentations page of the "Investors" section of the Company's website.

A replay of the webinar will be available upon conclusion of the webinar in the Investors section of the Editas Medicine website at [www.editasmedicine.com](http://www.editasmedicine.com).

#### **About Editas Medicine**

As a pioneering gene editing company, Editas Medicine is focused on translating the power and potential of the 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](http://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, including statements regarding the potential market for EDIT-401, if approved. 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 preclinical studies and its research and development programs, including the Company's expectation to achieve human proof-of-concept data for EDIT-401 by end of 2026 and identify and disclose an additional target cell type or tissue by the end of 2025; the potential of, and expectations for, EDIT-401; the timing or likelihood of regulatory filings and approvals, including submission of an IND or CTA for EDIT-401 by mid-2026; and the Company's expectations regarding its cash runway. 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 and completion of preclinical studies and clinical trials; availability and timing of results from preclinical studies and clinical trials; expectations for regulatory approvals to conduct trials; that the outcome of preclinical testing may not be indicative of the results of clinical trials and early clinical trials may not be predictive of the outcomes of later clinical trials; that preclinical data of one compound may not be comparable with clinical data for another compound; that the market opportunity for EDIT-401 may not be as significant as the Company expects; and uncertainties with respect to the availability of resources and financing sufficient to fund the Company's 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.