Editas Medicine Reports In Vivo Proof-of-Concept Data for EDIT-401 at the European Society of Gene and Cell Therapy (ESGCT) 32nd Annual Congress

October 9, 2025

Preclinical data demonstrates proof-of-concept for EDIT-401 with ≥90% mean LDL-C reduction in non-human primates and mouse models

CAMBRIDGE, Mass., Oct. 09, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company, today reported *in vivo* preclinical proof-of-concept data for EDIT-401, an experimental, potential best-in-class, one-time therapy to significantly reduce LDL-cholesterol (LDL-C), at the 32nd Annual European Society of Gene and Cell Therapy (ESGCT) Congress in Seville, Spain. The Company shared results from preclinical studies demonstrating potent and durable reductions in LDL-C through upregulation of the LDL receptor (LDLR).

Key EDIT-401 Data Presented includes:

- **Robust efficacy data:** ≥90% LDL-C reduction in non-human primates achieved within 48 hours of a single dose of EDIT-401; ≥90% LDL-C reduction in mice with high baseline LDL-C and reduced LDLR function
- **Optimized therapeutic strategy:**
  - CRISPR/Cas9 nuclease and dual gRNAs with LNP delivery disrupt negative regulatory elements in the 3' UTR, increasing mRNA stability enabling potent LDLR upregulation
  - ≥6-fold mean increase in LDLR protein in the NHP liver, requiring only a moderate level of functional editing of *LDLR* alleles
- **Durable effect:** LDL-C reduction maintained in mouse models in a three-month study

"The *in vivo* proof-of-concept data presented today reinforce the potential impact of our differentiated upregulation strategy. In preclinical non-human primate studies, EDIT-401 achieved robust efficacy data with a ≥90% mean LDL-C reduction. These data strengthen our conviction that EDIT-401 represents a novel therapeutic approach with the potential to significantly improve outcomes for people living with high LDL cholesterol," said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine.

Abstracts are available to registrants on the ESGCT website. The presentation will also be posted to the "Posters & Presentations" section of the Company's website at the time of the presentation and will remain accessible following the event.

Oral Presentation Details:

- **Title:** A transformative LDL cholesterol-lowering in vivo CRISPR gene editing medicine that functionally upregulates LDLR in mice and non-human primates
- **Session Date and Time:** Thursday, October 9, 5:00 p.m. CEST / 11:00 a.m. ET
- **Session Title:** 9A: Gene Editing II, Ex Vivo Applications
- **Room:** Parallel A
- **Presenter:** Linda Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine
- **Final Abstract Number:** OR069

About Editas Medicine

As a pioneering gene editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas12a and CRISPR/Cas9 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.

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