Editas Medicine Reports New In Vivo Proof of Concept Data in an Undisclosed Liver Target at the American Society of Gene and Cell Therapy Annual Meeting

May 13, 2025

In vivo *CRISPR Editing Results in Functional Upregulation of a Liver Target Protein and Meaningful Reduction of Disease-Associated Biomarker in Mice*

CAMBRIDGE, Mass., May 13, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company, today shared *in vivo* proof of concept data supporting the development of a potentially first-in-class treatment for an undisclosed liver target in a poster presentation at the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) in New Orleans. Editas scientists will present the data in a poster session on Wednesday, May 14, 2025, 5:30 p.m. – 7:00 p.m. CT (6:30 p.m. – 8:00 p.m. ET).

An *in vivo* editing strategy using lipid nanoparticles (LNPs) with CRISPR/Cas RNA cargo was employed for an undisclosed liver target gene. The strategy mimics a naturally occurring, protective variant, resulting in upregulation of the target gene. This resulted in meaningful reduction in the clinically relevant disease-specific biomarker in mice.

Key findings include:

- An *in vivo* dose-response study in a disease-specific mouse model utilizing LNPs to deliver CRISPR/Cas-based cargo demonstrated maximal liver editing of the target gene (~70%) and resulted in robust target protein upregulation with >80% disease biomarker reduction.
- Editing and subsequent upregulated expression of the target gene in cynomolgus monkey hepatocytes treated with CRISPR/Cas-based editing cargo also achieved >50% target gene editing and >15-fold protein upregulation.

“This *in vivo* proof of concept data in an undisclosed liver disease target confirms our ability to achieve maximal target gene editing within hepatocytes and clinically meaningful reduction in disease biomarkers. We believe this therapeutic approach will be transformative in the future treatment of this disease,” said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. “We are making significant progress towards the clinic and look forward to sharing the disease target and our development candidate later this year.”

Additional data on the undisclosed liver target will be shared in an oral presentation on May 21 at TIDES USA 2025: Oligonucleotide & Peptide Therapeutics Conference in San Diego.

Poster Presentation Details:
Title: *In Vivo* CRISPR Editing of Genetic Regulatory Regions Results in Functional Upregulation of Target Protein and Meaningful Reduction of Disease-Associated Biomarker in Mice
Session Date and Time: Wednesday, May 14, 2025, 5:30 p.m. – 7:00 p.m. CT
Session Title: Wednesday Poster Reception
Presentation Room: Poster Hall, Hall 12
Final Abstract Number: AMA351

Additional Editas Medicine presentations are below. Abstracts can be accessed on the ASGCT website, and the presentations will be posted on the Editas Medicine website during the conference.

Oral Presentation:
Title: *In Vivo* Delivery of HBG1/2 Promoter Editing Cargo to HSC of Humanized Mouse and Non-Human Primate with Lipid Nanoparticles
Session Date and Time: Wednesday, May 14, 2025, 1:30 p.m. – 1:45 p.m. CT
Session Title: Translational Applications of Base and Prime Editors
Room: 265-268
Final Abstract Number: AMA353

Poster Presentations:
Title: Design and Development of Improved LNP Targeting Ligands for *In Vivo* Hematopoietic Stem Cell Editing
Session Date and Time: Tuesday, May 13, 2025, 6:00 p.m. – 7:30 p.m. CT
Session Title: Tuesday Poster Reception
Presentation Room: Poster Hall, Hall 12
Final Abstract Number: AMA245

Title: Design of Chemically Modified AsCas12a Guide RNAs for Increased Potency of LNP-Delivered Gene Editing Cargos
Session Date and Time: Tuesday, May 13, 2025, 6:00 p.m. – 7:30 p.m. CT
Session Title: Tuesday Poster Reception
Presentation Room: Poster Hall, Hall 12
Final Abstract Number: AMA420

Title: *In Vivo* Gene Editing and Disease-Associated Biomarker Reduction for Multiple Liver Targets in Non-human Primate Using AsCas12a Nuclease Delivered by LNP

Session Date and Time: Wednesday, May 14, 2025, 5:30 p.m. – 7:00 p.m. CT
Session Title: Wednesday Poster Reception
Presentation Room: Poster Hall, Hall 12
Final Abstract Number: AMA640

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 *in vivo* medicines for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, 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.

Media and Investor Contact:
ir@editasmed.com

Source: Editas Medicine, Inc.