Editas Medicine to Present Preclinical Data Demonstrating Progress in the Development of an in vivo Gene Editing Pipeline at the American Society of Gene and Cell Therapy Annual Meeting

April 28, 2025

CAMBRIDGE, Mass., April 28, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company, today announced that five abstracts have been accepted for presentation, including one oral presentation, at the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) being held May 13 – 17, 2025, in New Orleans, LA, and virtually. The Company is presenting preclinical data to support its development of transformative *in vivo* gene editing medicines.

Editas Medicine presentations at ASGCT include:

- Oral presentation of *in vivo* preclinical data from humanized mouse and non-human primate (NHP) studies using targeted lipid nanoparticles (tLNPs) to deliver *HBG1/2* promoter editing cargo to hematopoietic stem and progenitor cells (HSPCs) and/or hematopoietic stem cells (HSCs) in bone marrow.
- Preclinical proof of concept for an undisclosed liver target using *in vivo* CRISPR editing to upregulate target protein expression and reduce a disease-associated biomarker in a relevant mouse disease model.
- Proof of concept results from the first *in vivo* mouse and NHP studies demonstrating high levels of target gene editing in the liver and corresponding biomarker response following intravenous administration of AsCas12a messenger RNA (mRNA) and chemically modified guide RNAs (gRNAs) delivered using LNPs from Genevant.
- Additional preclinical data demonstrating *in vivo* gene editing capabilities towards developing transformative *in vivo* medicines, including guide modification and targeting moiety optimizations to increase potency and improve gene editing outcomes *in vivo*.

“Editas Medicine is making significant progress towards the clinic with our *in vivo* medicines in preclinical development for people living with serious diseases,” said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. “We look forward to sharing further proof of concept data at ASGCT, including preclinical data confirming our ability to increase the level of a protein to address diseases caused by loss of function or deleterious mutations via our differentiated gene upregulation editing strategy. Our progress with tLNP delivery highlights the potential to execute our gene upregulation strategy across multiple tissues with our ‘plug ‘n play’ approach.”

The complete list of Editas Medicine presentations is 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

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

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.

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Source: Editas Medicine, Inc.