Editas Medicine Reports New In Vivo Data Highlighting the Potential of Editas' Gene Upregulation Strategy in HSCs at the American Society of Gene and Cell Therapy Annual Meeting

May 14, 2025

Data demonstrate therapeutically relevant editing levels using a clinically validated strategy, supporting its development as a novel, in vivo *approach to treating sickle cell disease and beta thalassemia*

CAMBRIDGE, Mass., May 14, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company, today shared new *in vivo* data demonstrating therapeutically relevant levels of *HBG1/2* promoter editing in hematopoietic stem cells (HSCs) with a single dose of proprietary targeted lipid nanoparticle (tLNP) in humanized mice and non-human primates (NHPs). This clinically validated approach targeting *HBG1/2* promoters to upregulate fetal hemoglobin (HbF) is in pre-clinical development as a potential transformative *in vivo* gene editing medicine for the treatment of sickle cell disease and beta thalassemia. The Company reported these data in a presentation available today and will detail the data in an oral presentation today at 1:30 p.m. CT/2:30 p.m. ET at the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) in New Orleans, LA, and virtually.

In these studies, the Company's proprietary tLNP formulation delivered *HBG1/2* promoter editing cargo to HSPCs and/or HSCs in humanized mice (mice engrafted with human CD34+ cells) and in NHPs. In an ongoing NHP study, administration of a single intravenous dose of Editas Medicine's proprietary tLNP demonstrated high efficiency HSC delivery and achieved up to 47% *HBG1/2* editing levels. In a study with humanized mice, administration of a single dose achieved 48% editing of *HBG1/2* in long-term HSCs. Both studies exceeded the predicted editing threshold of ≥25% required for therapeutic benefit. In addition to achieving therapeutically relevant editing levels, preliminary biodistribution data in NHPs with Editas' tLNP shows significant de-targeting of the liver in contrast to standard LNPs.

"These findings are very encouraging and further support our approach to developing a potentially first- and best-in-class *in vivo* gene edited medicine for the treatment of sickle cell disease and beta thalassemia," said Linda C. Burkly, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. "We believe that translating these preclinical results to the clinic will address the continuing significant unmet need for a transformative gene edited medicine with the potential to improve the lives of people living with sickle cell disease and beta-thalassemia around the world."

Editas Medicine's *in vivo* HSC program targets *HBG1/2* promoters to mimic naturally occurring mechanisms of hereditary persistence of fetal hemoglobin (HPFH) and utilizes proprietary AsCas12a to edit with high efficiency and minimize off-target editing. Editing the *HBG1/2* promoters with AsCas12a with the investigational medicine reni-cel led to robust increases in fetal hemoglobin (HbF) and total hemoglobin (Hb) in clinical trials.

Oral Presentation Details:
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

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.

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.

Media and Investor Contact:
ir@editasmed.com

Source: Editas Medicine, Inc.