



Editas Medicine Reports Proprietary Targeted Lipid Nanoparticle Delivery in Non-Human Primates Enables In Vivo HBG1/2 Promoter Editing for Sickle Cell Disease and Beta Thalassemia at the European Hematology Association 2025 Congress in June

June 12, 2025

Achieved 58% mean editing at five months after a single dose using high efficiency HSC delivery, demonstrating therapeutically relevant editing levels using a clinically validated strategy.

Achievement supports development of a novel, in vivo approach to treating sickle cell disease and beta thalassemia.

CAMBRIDGE, Mass., June 12, 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 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 a poster session on Saturday, June 14th 6:30 - 7:30 p.m. CEST (12:30 – 1:30 p.m. EDT) at the European Hematology Association (EHA) 2025 Congress in Milan, Italy.

In this study, the Company's proprietary tLNP formulation delivered *HBG1/2* promoter editing cargo to HSCs in NHPs. Latest data from this ongoing NHP study showed that at five months a single intravenous administration of Editas' tLNP resulted in mean on-target editing levels in the *HBG1/2* promoter region of 58% in HSCs: well exceeding the predicted editing threshold of $\geq 25\%$ required for therapeutic benefit. In addition to achieving therapeutically relevant editing levels, the biodistribution data in NHPs with Editas' tLNP continue to show significant de-targeting of the liver in contrast to standard LNPs.

"These data from our *in vivo* HSC program confirm our ability to achieve high efficiency delivery, therapeutically relevant editing levels and favorable biodistribution in NHPs. These data validate the further development of Editas' proprietary HSC-tLNP for editing of the *HBG1/2* promoters 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.

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 HbF and total hemoglobin (Hb) in clinical trials.

The presentation details are listed below. Abstracts can be accessed on the [EHA website](#), and the presentation will be posted on the [Editas Medicine website](#) during the conference.

Poster Presentation Details:

Title: Targeted Lipid Nanoparticle Delivery in Non-Human Primates Enables *In Vivo* *HBG1/2* Promoter Editing for β -hemoglobinopathies

Date/Time: Saturday, June 14, 2025, 6:30 - 7:30 p.m. CEST/ 12:30 – 1:30 p.m. EDT

Location: Allianz MiCo, Milano Convention Centre

Session: Poster Session 2

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.