Editas Medicine Announces Strategic Transition to in vivo Gene Editing Company with Intent to Achieve Human Proof of Concept in Approximately Two Years

December 12, 2024

- *Focus on* in vivo *CRISPR-edited medicines based on Editas researchers' recent scientific progress in multiple tissues:*
  - *Achieved pre-clinical* in vivo *proof of concept of high level HBG1/2 promoter editing and HbF induction in a humanized mouse model for treatment of sickle cell disease and beta thalassemia with a single dose of an HSC-targeted lipid nanoparticle (tLNP) formulation*
  - *Achieved* in vivo *proof of concept of high efficiency editing in the liver in non-human primates*

- *Ending development of reni-cel after extensive search did not yield a commercial partner*
  - *The Company will work closely with the clinical trial sites, regulators, and other parties to determine the path forward for patients enrolled in the RUBY and EdiTHAL trials*

- *Initiating cost savings measures and reduction in headcount to align workforce and resources to* in vivo *pipeline, extending cash runway into Q2 2027*

- *Conference call and webcast today at 5:00 p.m. ET*

CAMBRIDGE, Mass., Dec. 12, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading gene editing company, today announced a critical pivot to optimize its cost structure, extend its cash runway into Q2 2027, and position the Company to accelerate its intent to achieve *in vivo* human proof of concept in approximately two years.

"Recent scientific breakthroughs by the Editas team have convinced us that the timelines around the near-term viability of *in vivo* CRISPR-edited medicines have accelerated meaningfully. Two years ago, we laid out our strategy and objective to become a leader in *in vivo* programmable gene editing. Based on these advances, we are transitioning to a fully *in vivo* company. We believe the ability to provide *in vivo* gene editing that functions via gene upregulation across tissues holds the potential to significantly expand the addressable therapeutic possibilities for CRISPR-based gene editing and uniquely position Editas to be a leader in the field moving forward," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine.

The Company transition follows the recent *in vivo* pre-clinical proof of concept in multiple tissues:

- **Hematopoietic Stem Cells (HSCs):**
  - Editas achieved ~40% editing of the *HBG1/2* promoter site after using a novel, Editas-proprietary targeted lipid nanoparticle (tLNP) for extrahepatic tissue delivery to deliver a single dose of its clinically validated Cas12a editing machinery directly to human hematopoietic stem cells (HSCs) in mice engrafted with human HSCs.[1]
  - *HBG1/2* biology has been validated and derisked in patients with reni-cel in the RUBY trial.
  - The editing in HSCs with the Company's proprietary tLNP formulation resulted in the meaningful functional outcome of HbF induction, indicated by the presence of HbF expressing human red blood cells (on average 20%) that populate in the host by one month.

- **Liver:**
  - The Company achieved *in vivo* proof of concept of high efficiency editing in the liver in non-human primates under its collaboration with Genevant.

The Company intends to share pre-clinical data and further development timelines from these programs in the first quarter of 2025.

In vivo HSC editing success is expected to enable extrahepatic tissues/cell types targeting beyond HSCs and demonstrates the potential of "plug 'n play" in an *in vivo* extrahepatic LNP platform. The Company's upregulation capability additionally enables a differentiated strategy for liver targets for diseases with high unmet need and first-in-class opportunities.

In connection with Editas Medicine's transition to an *in vivo* company, the Company initiated a reduction in headcount that will eliminate approximately 65% of its workforce over the next six months. As part of this reduction in force, several members of the Editas management team will depart the company over the next six months, including Baisong Mei, M.D., Ph.D., the Company's Chief Medical Officer.

Additionally, Emma Reeve and Meeta Chatterjee, Ph.D. are resigning from the Board of Directors, effective December 31, 2024. Jessica Hopfield, Ph.D., has been named Chair of the Board, effective December 31, 2024.

Dr. O'Neill added, "We want to extend our deepest appreciation to patients, investigators, clinical sites staff, and our employees who have shown tremendous dedication and commitment to developing a potentially transformational medicine like reni-cel. We also want to express specific gratitude to the patients in our clinical trials and their caregivers whose dedication to disease research for their community makes us even more committed to

accelerating our efforts towards an *in vivo* program for sickle cell disease and beta thalassemia."

Conference Call
The Editas Medicine management team will host a conference call and webcast today at 5:00 p.m. ET. To access the call, please dial 1-877-407-0989 (domestic) or +1 201-389-0921 (international) and ask for the Editas Medicine conference call. A live webcast of the call will also be available on the Investors section of the Editas Medicine website at www.editasmedicine.com, and a replay will be available approximately two hours after its completion.

About Editas Medicine
As a leading 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.

Forward-Looking Statements
This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "should,'' ''would,'' and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include statements regarding the Company's transition to a fully *in vivo* company, the intention to achieve human proof of concept in approximately two years, and the potential success of its *in vivo* gene editing programs, the timing for releasing additional pre-clinical data, the anticipated effects, including potential cost savings, of the Company's decision to discontinue development of reni-cel and initiate the related reduction in headcount, the scope and timing of the reduction in headcount, and the expected extension of the Company's cash runway. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of preclinical studies; availability and timing of results from preclinical studies; expectations for regulatory approvals to conduct trials; availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; and that the decision to discontinue clinical development of reni-cel and the related reduction in headcount may have unexpected consequences or not result in the expected cost savings. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by the Company's subsequent filings with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

[1] Previously disclosed editing of 29% in hematopoietic stem and progenitor cell (HSPCs) at one week after a single dose in a Strategic Update webinar in October 2024.

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Source: Editas Medicine, Inc.