Editas Medicine Announces Progress Towards 2024 Goals, Including Achievement of In Vivo Preclinical Proof of Concept and Strategic Update

October 22, 2024

Achieved in vivo *preclinical proof of concept of hematopoietic stem and progenitor cell editing by utilizing Editas Medicine's proprietary targeted LNP as a key step forward toward developing a novel* in vivo *treatment for sickle cell disease and beta thalassemia*

Initiated process to partner or out-license reni-cel to focus resources on in vivo *pipeline development*

Company to present data and discuss strategic update in a Company-sponsored Webinar today at 8:00 a.m. ET

CAMBRIDGE, Mass., Oct. 22, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage gene editing company, today announced its achievement of *in vivo* preclinical proof of concept of hematopoietic stem and progenitor cell (HSPC) editing and fetal hemoglobin (HbF) induction in humanized mice engrafted with human hematopoietic stem cells and lacking their own hematopoietic cells. The Company observed high levels of editing of the HBG1/2 promoter, leveraging our clinically validated upregulation strategy, utilizing a novel and Editas-proprietary targeted lipid nanoparticle (tLNP) formulation for extrahepatic tissue delivery. The Company is also providing business development and financial updates, including that it initiated a process to partner or out-license reni-cel.

"Achieving *in vivo* preclinical proof of concept in a desired, extrahepatic target cell type, delivered utilizing a proprietary Editas LNP that works outside the liver, puts us on a clear path to develop a potentially first- and best-in-class *in vivo* gene edited medicine for the treatment of sickle cell disease and beta thalassemia," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "I am delighted with our concrete progress on *in vivo* functional upregulation as we drive towards our long-term vision to be a leader in *in vivo* programmable gene editing, specifically achieving *in vivo* preclinical proof of concept ahead of schedule."

Dr. O'Neill added, "Simultaneously, we are laser focused on the optimal use of capital as we continue development of reni-cel, invest in our *in vivo* pipeline and vision, and map to the future. As part of that focus, we frequently evaluate opportunities to most efficiently advance reni-cel, a potentially best-in-class cell therapy for the treatment of sickle cell disease and beta thalassemia, towards commercialization and ultimately deliver it to patients in need. We believe that the best option for both patients and our shareholders is for us to seek an alternative such as a global partner or out-licensing, which would allow for further development and ultimately commercialization of reni-cel with or by another party and would allow us to substantially reduce spend in 2025."

***In Vivo* Proof of Concept Data Highlights**

- Achieved highly competitive level of *in vivo* hematopoietic stem and progenitor cell (HSPC) editing utilizing a novel, Editas-proprietary targeted lipid nanoparticle (t-LNP) for extrahepatic tissue delivery in a humanized mouse model, mice engrafted with human hematopoietic stem cells.
- Enabled an editing level of 29% in HSPCs after a single dose by utilizing a hematopoietic stem cell targeting strategy and tLNP formulation optimization.
- Further demonstrated that editing with the Company's proprietary tLNP formulation resulted in the 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.
- This level of *in vivo* editing in a humanized mouse model after a single dose constitutes a highly competitive dataset relative to data in the public domain for the development of an *in vivo* medicine for sickle cell disease and beta thalassemia.

***Ex Vivo* Hemoglobinopathies**

- **Reni-cel (renizgamglogene autogedtemcel, previously EDIT-301) for Severe Sickle Cell Disease (SCD)**
  - On-track to present a substantive clinical data set of sickle cell patients with considerable clinical follow-up in the RUBY trial at the American Society of Hematology (ASH) Annual Meeting and Exposition, December 7-10, 2024.
  - As previously disclosed, the Company has completed enrollment of the adolescent and adult cohorts of the Phase 1/2/3 RUBY trial for SCD.
  - Manufacturing drug product for the initial adolescent cohort patients.
  - The Company continues to dose adult patients in the RUBY trial and has dosed 28 patients to date.
- **Reni-cel for Transfusion-dependent Beta Thalassemia (TDT)**
  - On-track to present additional clinical data from the EdiTHAL trial by year-end 2024.
  - The Company completed enrollment of the adult cohort of the EdiTHAL trial for TDT and continues to dose patients.

Other Corporate Highlights

- On October 3, 2024, Editas Medicine announced the sale of certain future license fees and other payments owed to the Company under its Cas9 license agreement with Vertex Pharmaceuticals to a wholly owned subsidiary of DRI Healthcare Trust (DRI) for an upfront cash payment of $57 million. The upfront cash payment brings non-dilutive capital to Editas Medicine, helping enable further pipeline development and related strategic priorities.
- The Company ended the third quarter 2024 with approximately $265 million of cash, cash equivalents, and marketable securities, or approximately $320 million following receipt of the upfront cash payment from DRI.
- The Company has engaged Moelis & Company LLC, a leading global independent investment bank, to lead the global process to partner or out-license reni-cel.

In light of this webinar, the Company does not plan to host a conference call when it announces third quarter 2024 financial results next month.

Webinar Presentation Details:
The live and archived webcast of the Company's webinar presentation will be accessible through this **webcast link**, or through the Events & Presentations page of the "Investors" section of the Company's website.

A replay of the webinar will be available upon conclusion of the webinar in the Investors section of the Editas Medicine website at https://www.editasmedicine.com/.

About renizgamglogene autogedtemcel (reni-cel)
Reni-cel, formerly known as EDIT-301, is an experimental gene editing medicine under investigation for the treatment of severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT). Reni-cel consists of patient-derived $CD34^+$ hematopoietic stem and progenitor cells edited at the gamma globin gene (*HBG1* and *HBG2*) promoters, where naturally occurring fetal hemoglobin (HbF) inducing mutations reside, by AsCas12a, a novel, proprietary, highly efficient, and specific gene editing nuclease. Red blood cells derived from reni-cel $CD34^+$ cells demonstrate a sustained increase in fetal hemoglobin production, which has the potential to provide a one-time, durable treatment benefit for people living with severe SCD and TDT.

About Editas Medicine
As a clinical-stage 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 treatments for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, durable, precision genomic 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 intent to partner or out-license reni-cel and any benefits resulting therefrom, the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including presenting additional clinical data from the RUBY trial at the ASH Annual Meeting and Exposition and from the EdiTHAL trial by year-end 2024, the potential of, and expectations for, the Company's product candidates, including any *in vivo* gene edited medicines the Company may develop, and the Company's expectations regarding 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 and clinical trials, including the RUBY and EdiTHAL trials, and clinical development of the Company's product candidates, including reni-cel; availability and timing of results from pre-clinical studies and clinical trials; whether interim results from preclinical studies and clinical trials will be predictive of the final results of the study or trial or the results of any future clinical trials; expectations for regulatory approvals to conduct trials or to market products; and availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements. 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.

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