Editas Medicine Granted FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for EDIT-301 for the Treatment of Severe Sickle Cell Disease

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CAMBRIDGE, Mass., Oct. 16, 2023 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage genome editing company, today announced that the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to EDIT-301, an investigational, gene editing medicine, for the treatment of severe sickle cell disease (SCD).

"Sickle cell disease is a devastating disease that leads to anemia, pain crises, organ failure, and early death. Receiving RMAT designation for EDIT-301 for severe sickle cell disease highlights the urgent need for new treatment options for patients and supports our belief that EDIT-301 can provide life-changing clinical benefits to patients," Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "I would like to thank the participants, their families, clinicians, and colleagues at collaborating institutions that contribute to the RUBY trial. We look forward to sharing further clinical updates including additional data for the trial prior to year-end."

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the development and review processes for promising regenerative medicine therapies. An investigational cell therapy medicine or gene editing medicine is eligible for RMAT designation if it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the experimental medicine has the potential to address unmet medical needs for the disease or condition. Advantages of the RMAT designation include all the benefits of the fast track and breakthrough therapy designation programs, including but not limited to intensive FDA guidance on efficient and expedited drug development, possible rolling review, and priority review of the biologics license application (BLA), and FDA's organizational commitment involving senior managers.

The FDA previously granted Orphan Drug Designation and Rare Pediatric Disease designation to EDIT-301 for the treatment of sickle cell disease and beta thalassemia.

About Sickle Cell Disease
Sickle cell disease is an inherited blood disorder caused by a mutation in the beta-globin gene that leads to polymerization of the sickle hemoglobin (HbS). In sickle cell disease, the red blood cells are misshapen in a sickle shape instead of a typical disc shape. The abnormal shape causes the red blood cells to have shortened lifespan and to block blood flow causing anemia, pain crises, organ failure, and early death. There are an estimated 100,000 people in the United States currently living with sickle cell disease. Higher levels of fetal hemoglobin (HbF) inhibit HbS polymerization, thus reducing the manifestation of sickling.

About EDIT-301
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). EDIT-301 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 a highly specific and efficient proprietary engineered AsCas12a nuclease. Red blood cells derived from EDIT-301 $CD34^+$ 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 the RUBY Trial
The RUBY trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in patients with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301. Additional details are available on www.clinicaltrials.gov (NCT#04853576).

About Editas Medicine
As a clinical-stage genome 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 expectation to provide further clinical data updates, including additional data for the RUBY trial by year-end. 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 trial, and clinical development of the Company's product candidates, including EDIT-301; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future 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.

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