Editas Medicine Receives FDA Orphan Drug Designation for EDIT-301 for the Treatment of Sickle Cell Disease

April 27, 2023

On track to provide clinical update for RUBY trial by mid-2023 and dose 20 total patients by year-end

CAMBRIDGE, Mass., April 27, 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 Orphan Drug Designation to EDIT-301, an investigational, gene editing medicine, for the treatment of sickle cell disease. The FDA previously granted Orphan Drug Designation to EDIT-301 for the treatment of beta thalassemia and Rare Pediatric Disease designation to EDIT-301 for the treatment of beta thalassemia and sickle cell disease.

"Sickle cell disease is a devastating disease that leads to anemia, pain crises, organ failure, and early death. Receiving Orphan Drug Designation for EDIT-301 for sickle cell disease highlights the urgent need for new treatment options for patients and supports our belief that EDIT-301 can be a clinically differentiated, one-time, durable medicine that 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 and clinical data for the trial in the near future."

The FDA's Orphan Drug Designation program provides orphan status to drugs or biologics intended for the prevention, diagnosis, or treatment of diseases that affect fewer than 200,000 people in the United States. Sponsors of medicines that are granted Orphan Drug Designation are entitled to certain incentives, including tax credits for qualified clinical trials, prescription drug user-fee exemptions, and potential seven-year marketing exclusivity upon FDA approval.

EDIT-301 is currently being investigated in a clinical study in patients with severe sickle cell disease (RUBY trial, NCT04853576) and transfusion-dependent beta thalassemia (EDITHAL trial, NCT#05444894). Editas Medicine will present clinical data updates from the RUBY trial twice this year, mid-year and again by year-end. Additionally, the Company is on-track to dose 20 patients in the RUBY trial by year-end.

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 cell therapy 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 RUBY
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/Cas9 and CRISPR/Cas12a 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 and Harvard University's Cas9 patent estates and Broad Institute's Cas12a patent estate 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 clinical data updates for the RUBY trial by mid-2023 and again by year-end and to dose 20 total patients 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, 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.

Media and Investor Contact:
Cristi Barnett
(617) 401-0113
cristi.barnett@editasmed.com

Source: Editas Medicine, Inc.