Editas Medicine Receives FDA Orphan Drug Designation for EDIT-301 for the Treatment of Beta Thalassemia

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Company on track to dose first transfusion-dependent beta thalassemia patient with EDIT-301 by year-end

CAMBRIDGE, Mass., May 12, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading 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 beta thalassemia. The FDA previously granted Rare Pediatric Disease designation to EDIT-301 for the treatment of beta thalassemia and sickle cell disease.

“Beta thalassemia is a devastating disease that leads to severe anemia, organ failure, and premature death. Receiving Orphan Drug Designation for EDIT-301 for beta thalassemia highlights the urgent need for new treatment options for patients,” said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. “Preparations to initiate the Phase 1/2 clinical trial of EDIT-301, a potentially transformative medicine for people living with beta thalassemia, are underway, and we look forward to dosing the first patient in the clinical trial this year.”

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). Editas expects to initiate a Phase 1/2 study of EDIT-301 in patients with transfusion-dependent beta thalassemia in 2022.

About Beta Thalassemia

Beta thalassemia is a common autosomal recessive disorder with an estimated annual incidence rate of 1 in 100,000 worldwide for symptomatic individuals. Beta thalassemia mutations reduce or abrogate beta globin expression. Insufficient beta globin production leads to ineffective red blood cell production, chronic hemolytic anemia due to the destruction of red blood cells, and compensatory extramedullary hematopoiesis (creation of blood cells). Based on clinical severity and transfusion requirements, beta thalassemia can be classified into non-transfusion-dependent (NTDT) and transfusion-dependent beta thalassemia (TDT). TDT is the most severe form of beta thalassemia, and patients require lifelong regular red blood cell transfusions to prevent organ failure and death. Chronic red blood cell transfusions are complicated by iron overload leading to organ dysfunction and failure. Left untreated, the mortality rate among TDT patients is high, with a survival rate of only 15 percent at age five due to severe anemia.

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 sickle cell disease and transfusion-dependent beta thalassemia.

About Editas Medicine

As a leading 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. 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 initiate a Phase 1/2 study of EDIT-301 in patients with TDT in 2022 and dosing the first patient in the TDT clinical trial this year. 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 factors, including: uncertainties inherent in the initiation and completion of pre-clinical studies and clinical trials and clinical development of the Company’s product candidates; availability and timing of results from pre-clinical 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 represent the Company’s views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.

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