Editas Medicine Announces FDA Clearance of Investigational New Drug (IND) Application for EDIT-301 for the Treatment of Transfusion-Dependent Beta Thalassemia

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EDIT-301 is in development as a transformative, one-time treatment for people living with transfusion-dependent beta thalassemia

Editas Medicine will initiate a Phase 1/2 clinical trial in 2022

CAMBRIDGE, Mass., Dec. 20, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced that the U.S. Food and Drug Administration (FDA) has cleared the IND for EDIT-301 for the treatment of transfusion-dependent beta thalassemia (TDT), enabling the Company to initiate a Phase 1/2 clinical study of EDIT-301 in TDT patients.

EDIT-301 is an experimental gene editing medicine designed to be a transformative, one-time treatment for people living with severe sickle cell disease (SCD) or TDT. EDIT-301 uses CRISPR/Cas12a gene editing technology to precisely make a DNA change that restores production of fetal hemoglobin (HbF), which is usually only present early in life. Some people with SCD or TDT also have a natural DNA change called hereditary persistence of fetal hemoglobin (HPFH), and in these people, HbF remains into adulthood and protects them from severe forms of the disease and related complications.

"The FDA's clearance of our IND for EDIT-301 for TDT is an important milestone for Editas and for the patients we hope to serve. We believe that EDIT-301 has the potential to transform the lives of people living with TDT with our unique editing approach that may improve red blood cell production and eliminate transfusion burden, leading to better outcomes for patients," said James Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine.

With this IND clearance, Editas Medicine is preparing to initiate a Phase 1/2 trial designed to assess the safety, tolerability, and preliminary efficacy of EDIT-301 for the treatment of TDT.

Mr. Mullen added, "Ending 2021 with three programs in clinical development is a significant achievement for Editas, and we are thrilled to continue this momentum in creating next generation medicines."

About Transfusion-Dependent Beta Thalassemia (TDT)
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. Based on clinical severity and transfusion requirements, beta thalassemia can be classified into non-transfusion-dependent (NTDT) and transfusion-dependent beta thalassemia. 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 CRISPR/Cas12a ribonucleoprotein (RNP). 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 (SCD) and transfusion-dependent beta thalassemia (TDT).

EDIT-301 is currently being investigated in a clinical study in patients with severe SCD (RUBY trial, NCT04853576). With this IND clearance, Editas will initiate a new Phase 1/2 study of EDIT-301 in patients with TDT.

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 (also known as Cpf1) 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 initiating the Phase 1/2 clinical trial for EDIT-301 for the treatment of TDT in 2022. 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 preclinical studies and clinical trials and clinical development of the Company's product candidates; 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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