Editas Medicine Receives Rare Pediatric Disease Designation for EDIT-301 for the Treatment of Sickle Cell Disease

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CAMBRIDGE, Mass., Aug. 24, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation for EDIT-301, an experimental, autologous cell medicine, being developed as a potentially best-in-class, durable medicine for sickle cell disease. The Company plans to file an investigational new drug application (IND) for EDIT-301 by the end of 2020.

“The Editas team has a bold vision to unlock the potential of CRISPR to design and develop game-changing medicines. We are making tremendous progress towards this vision with the continued development of EDIT-301, a potentially transformative medicine for the treatment of sickle cell disease, and we are pleased to receive Rare Pediatric Disease designation from the FDA for this program,” said Cynthia Collins, Chief Executive Officer, Editas Medicine. “We know patients are counting on us, and this designation is a significant milestone for the program that highlights the serious, life-threatening manifestations of sickle cell disease.”

The FDA defines a rare pediatric disease as a serious or life-threating disease in which the serious or life-threatening disease manifestations primarily affect individuals aged from birth to 18 years. Pediatric diseases recognized as “rare” affect under 200,000 people in the United States. Under the FDA’s Rare Pediatric Disease Designation and Voucher Programs, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may be eligible for a voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product.

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 protein (HbS). In sickle cell disease, the red blood cells are misshapen, in a sickle shape instead of the disc shape. The abnormal shape causes the cells 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. Fetal hemoglobin (HbF) protects against sickle cell disease by inhibiting HbS polymerization.

About EDIT-301
EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) to edit the HBG1/2 promoter region in the beta-globin locus. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin (HbF) production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

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/Cpf1 (also known as 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 plans with respect to timing of filing an IND for EDIT-301 by the end of 2020. 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 Quarterly Report on Form 10-Q, 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 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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