Editas Medicine Announces Submission of IND Application for EDIT-301 with the FDA

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EDIT-301 is in development as a best-in-class, durable medicine for people living with sickle cell disease

CAMBRIDGE, Mass., Dec. 09, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced it submitted an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) for the initiation of a Phase 1/2 clinical trial of EDIT-301, an experimental CRISPR/Cas12a gene editing medicine in development for the treatment of sickle cell disease. The Company previously received Rare Pediatric Disease designation from the FDA for EDIT-301.

"This IND submission is a key milestone for Editas as we continue to advance several *ex vivo* cell therapy medicines. This submission brings us one step closer to entering the clinic with our potentially best-in-class, transformative, and durable medicine for people living with sickle cell disease," said Cynthia Collins, Chief Executive Officer, Editas Medicine. "This moment is very exciting for the Editas team. We know patients are counting on us, and we look forward to next steps for the clinical development of EDIT-301, including dosing sickle cell disease patients."

Editas Medicine continues to prepare for a Phase 1/2 clinical trial evaluating EDIT-301 for the treatment of sickle cell disease. The Company has identified a lead principal investigator and engaged a Clinical Research Organization (CRO). Clinical trial materials are being manufactured by Editas Medicine.

About Sickle Cell Disease
Sickle cell disease is caused by a mutation in the beta-globin gene that leads to polymerization of the sickle hemoglobin protein (HbS). Fetal hemoglobin (HbF) protects against sickle cell disease by inhibiting HbS polymerization. Individuals with high levels of HbF are protected from sickle cell disease. EDIT-301 is an experimental, autologous cell therapy comprising CD34+ cells genetically modified using a Cas12a ribonucleoprotein (RNP) that targets the *HBG1/2* promoter in the beta-globin gene to stimulate HbF production.

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 and expectations for EDIT-301. 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 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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