Editas Medicine Demonstrates Dose-Dependent, In Vivo Editing with EDIT-101 in CEP290 Transgenic Mice

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Productive gene editing rates with EDIT-101 were stable over six months in transgenic mice

CAMBRIDGE, Mass., Oct. 19, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced results from a pre-clinical study in transgenic mice demonstrating dose-dependent, *in vivo* editing using EDIT-101, Editas Medicine's pre-clinical product candidate for the treatment of Leber Congenital Amaurosis type 10 (LCA10). The study was conducted in mice that have a human CEP290 common intron 26 knock-in (HuCEP290 IVS26 KI mice), an animal model for most common genetic change that causes LCA10. The results of this study further reinforce Editas Medicine's belief in the transformative potential of EDIT-101 as a genome editing medicine to help patients with LCA10. LCA10 is an inherited retinal degenerative disease caused by mutations in the CEP290 gene that appears in childhood and leads to blindness. The Company reported these data today in a poster presentation at the 25th Anniversary Congress of the European Society of Gene and Cell Therapy (ESGCT) in Berlin.

In this study, HuCEP290 IVS26 KI mice were treated with EDIT-101 by subretinal injection, resulting in efficient transduction and gene editing in the retinal photoreceptor cells of the mice, which is the cell type affected in LCA10 patients. The onset of CEP290 gene editing was rapid and was detectable as early as three days post-delivery with a further significant increase in editing observed by one week. The components were measured, and both Cas9 mRNA and guide RNA (gRNA) levels correlated with editing. At the administered dose of 1E+12 vg/mL vector concentration, editing levels exceeded levels predicted to be therapeutically relevant in 90 percent (27/30) of EDIT-101 treated eyes with a median of 31 percent of photoreceptors harboring productively edited CEP290 in the transduced neuroretina. The productive CEP290 gene editing rates were stable through six months of observation, and the expression of Cas9 mRNA and gRNA decreased over time.

"To date, we have made significant progress in our LCA10 program. We have achieved predictive therapeutic levels of productive CEP290 gene editing in several pre-clinical settings, including in neural retinas in human cells, in transgenic mice, and in non-human primates. Collectively, these data demonstrate that we can deliver EDIT-101 to photoreceptors and can edit in the eye at levels well above the anticipated minimum therapeutic threshold, supporting the clinical development of EDIT-101 for the treatment of patients suffering from LCA10," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine.

Editas Medicine plans to submit an Investigational New Drug (IND) application for EDIT-101 in mid-2018. In March, Editas Medicine and Allergan Pharmaceuticals International Limited ("Allergan") entered into a strategic research and development alliance under which Allergan received an exclusive option to license up to five of Editas Medicine's genome-editing ocular programs, including Editas Medicine's lead program for LCA10. The agreement covers a range of first-in-class ocular programs targeting serious, vision-threatening diseases based on Editas Medicine's unparalleled CRISPR genome editing platform, including CRISPR/Cas9 and CRISPR/Cpf1.

About Leber Congenital Amaurosis

Leber Congenital Amaurosis (LCA) is a group of inherited retinal degenerative disorders caused by mutations in at least 18 different genes. It is the most common cause of inherited childhood blindness, with an incidence of two to three per 100,000 live births worldwide. Symptoms of LCA appear within the first year of life, resulting in significant vision loss and blindness. The most common form of the disease, LCA10, is a monogenic disorder caused by mutations in the CEP290 gene and is the cause of disease in approximately 20-30 percent of all LCA patients.

About Editas Medicine

Editas Medicine is a leading genome editing company dedicated to treating patients with genetically-defined diseases by correcting their disease-causing genes. The Company was founded by world leaders in genome editing, and its mission is to translate the promise of genome editing science into a broad class of transformative genomic medicines to benefit the greatest number of patients. To learn more about Editas Medicine, 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 goal of submitting of an IND for the LCA10 program by mid-2018 and translating the Company's CRISPR technology into medicines to help patients suffering from LCA10. 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 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 as a result of new information, future events or otherwise.

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