Editas Medicine Demonstrates First Achievement of In Vivo Editing in Non-human Primate Retinas

May 13, 2017 11:05 AM ET

Editing rate reported at ASGCT has potential to translate into a transformative therapy for LCA10 patients

CAMBRIDGE, Mass., May 13, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced results from a pre-clinical study demonstrating the first achievement of efficient editing of the CEP290 gene in the retina of non-human primates. The results of this study further reinforce Editas Medicine's belief in the transformative potential of its candidate medicine for Leber Congenital Amaurosis type 10 (LCA10), an inherited retinal degenerative disease that appears in childhood and leads to blindness. The Company reported these data today in an oral presentation at the 20th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) in Washington, D.C.

In this study, two guide RNAs and *S. aureus* Cas9 expressed under the control of a photoreceptor-specific promoter were delivered in a single, subretinally-injected adeno-associated virus (AAV) at two different doses. Retinal tissue and genomic DNA were taken from within the sub-macular bleb region at six and 13 weeks and analyzed using Editas Medicine's novel sequencing method, UDiTaS™, to accurately quantify all editing events. Gene editing was demonstrated to be dose and time dependent. Editing rates were measured directly as a percentage of all alleles in total genomic DNA taken from the retinas of non-human primates, which included both photoreceptor cells and other cell types. Editing rates were also projected based on additional analyses which demonstrated that Cas9 expression was limited to photoreceptor cells, which are the target cells for LCA10 treatment. For animals treated with the higher dose, the projected productive editing rate may be as high as 50 percent in photoreceptor cells, based on directly measured editing of 15 percent in total genomic DNA and an estimate for the proportion of cells represented by photoreceptors. Achieving 50 percent of all alleles would be well above the editing rate hypothesized to have a therapeutic effect in patients.

"Based on analyses presented today, we believe that we were successful in editing the vast majority photoreceptors using a primate-specific candidate that shares critical design and construction features with our clinical candidate," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "These data are an important step towards the clinic and substantially increase our confidence that we can translate CRISPR technologies into medicines to help patients suffering from this devastating eye disease."

In March, Editas Medicine and Allergan entered into a strategic research and development alliance under which Allergan received exclusive access and the option to license up to five of Editas Medicine's genome-editing candidate medicines for ocular diseases, including its product candidate for LCA10. The agreement covers a range of first-in-class ocular programs targeting serious diseases based on Editas Medicine's unparalleled CRISPR genome editing platform, including CRISPR/Cas9 and CRISPR/Cpf1.

About Leber Congenital Amaurosis 10
Leber Congenital Amaurosis, or LCA, is a group of inherited retinal dystrophies 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, referred to as LCA10, is a monogenic disorder caused by mutations in the CEP290 gene and represents approximately 20-30 percent of all LCA subtypes.

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.

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,” similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. 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, 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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