



Clinical Data from Editas Medicine's Ongoing Phase 1/2 BRILLIANCE Clinical Trial of EDIT-101 for LCA10 to be Presented at the European Society of Gene and Cell Therapy Annual Congress

October 20, 2021

CAMBRIDGE, Mass., Oct. 20, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced that an abstract featuring initial clinical data from the BRILLIANCE clinical trial of EDIT-101 has been selected for an oral presentation at the 2021 European Society of Gene and Cell Therapy (ESGCT) Annual Congress being held virtually October 19 – 22, 2021. EDIT-101 is under development for the treatment of Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder.

"We are pleased to be working at the forefront of this research with Editas Medicine, evaluating genome editing for the treatment of *CEP290*-associated retinal disease in the BRILLIANCE trial," said Eric A. Pierce, M.D., Ph.D., Director of the Ocular Genomics Institute and William F. Chatlos Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School, and the senior BRILLIANCE principal investigator. "I am highly encouraged by the early efficacy signals in the mid-dose cohort, which suggest positive biological activity and potential early clinical benefits. I am also very pleased that the initial data from the BRILLIANCE trial demonstrate a favorable safety profile. I believe that the trial data support continued EDIT-101 development as well as the evaluation of gene editing approaches for other inherited retinal disorders."

Details of the Editas Medicine presentation can be accessed on the [ESGCT website](#).

Oral Presentation:

Title: BRILLIANCE: A Phase 1/2 single ascending dose study of EDIT-101, an in vivo CRISPR gene editing therapy, in *CEP290*-related retinal degeneration

Session Title: Parallel 4b: Gene editing II

Date and Time: Thursday, October 21, 2021, 10:15 – 10:30 a.m. CET

Presenter: Dr. Eric A. Pierce, M.D., Ph.D., Director of the Inherited Retinal Disorders Service, Director of the Ocular Genomics Institute and William F. Chatlos Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School, and a BRILLIANCE principal investigator.

About EDIT-101

EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder. EDIT-101 is administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells.

About BRILLIANCE

The BRILLIANCE Phase 1/2 clinical trial of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder, is designed to assess the safety, tolerability, and efficacy of EDIT-101 in up to 18 patients with this disorder. Clinical trial sites are enrolling up to five cohorts testing up to three dose levels in this open label, multi-center study. Both adult and pediatric patients (3 – 17 years old) with a range of baseline visual acuity assessments are eligible for enrollment. Patients receive a single administration of EDIT-101 via subretinal injection in one eye. Patients are monitored every three months for a year after dosing and less frequently for an additional two years thereafter. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

About Leber Congenital Amaurosis

Leber Congenital Amaurosis, or 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 approximately three per 100,000 live births worldwide. Symptoms of LCA appear within the first years of life, resulting in significant vision loss and potentially blindness. The most common form of the disease, LCA10 or a *CEP290*-related retinal degenerative disorder, 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

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. 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 completion of clinical trials, including the BRILLIANCE trial, 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 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 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.

Contacts:

Media

Cristi Barnett

(617) 401-0113

cristi.barnett@editasmed.com

Investors

Ron Moldaver

(617) 401-9052

ir@editasmed.com



Source: Editas Medicine, Inc.