



## **Editas Medicine Announces Enrollment of the First Pediatric Cohort in the BRILLIANCE Clinical Trial of EDIT-101 for the Treatment of LCA10 Following IDMC Endorsement**

June 23, 2021

*IDMC endorsed proceeding with first pediatric cohort based on a review of clinical safety data from adult low-dose and adult mid-dose cohorts*

*Concurrently enrolling the adult high-dose cohort in the BRILLIANCE trial*

*Company plans to report initial EDIT-101 clinical data in September*

CAMBRIDGE, Mass., June 23, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced that centers are now enrolling the first of two planned pediatric cohorts in the Phase 1/2 BRILLIANCE clinical trial of EDIT-101 following the Independent Data Monitoring Committee (IDMC) endorsement to proceed. The IDMC endorsement is based on an analysis of safety data from the adult low-dose and adult mid-dose cohorts. The Company also announced the completion of dosing of the adult mid-dose cohort. Both the pediatric mid-dose cohort and the adult high-dose cohort will run concurrently. For both cohorts, first dosing is expected this summer and completion is expected in the first half of 2022. EDIT-101 is under development for the treatment of Leber congenital amaurosis 10 (LCA10).

"We are extremely pleased with the endorsement of the IDMC, and we look forward to bringing EDIT-101 to additional patients in the BRILLIANCE trial. LCA10 is an early onset retinal degenerative disease resulting in significant vision loss and blindness. Positive feedback from the IDMC is a critical step to move to initiate EDIT-101 dosing in pediatric patients," said Lisa Michaels, M.D., Executive Vice President and Chief Medical Officer, Editas Medicine. "I would like to thank all of the patients who have and will participate in this landmark gene editing medicine clinical trial. We are planning to report initial clinical data at a medical meeting this September."

"We have a long history at Mass. Eye and Ear of helping develop life-changing medicines, and we are pleased to be working at the forefront of research on this novel therapy. We are eager to begin studying EDIT-101 in pediatric patients where we believe it can have great impact and the potential to restore vision," said Eric A. Pierce, M.D., Ph.D., Director of the Inherited Retinal Disorders Service and Director of the Ocular Genomics Institute at Massachusetts Eye and Ear, the William F. Chatlos Professor of Ophthalmology at Harvard Medical School, and a Principal Investigator for the BRILLIANCE clinical trial.

Mark Pennesi, M.D., Ph.D., Associate Professor of Ophthalmology, Kenneth C. Swan Endowed Professor, Division Chief, Paul H. Casey Ophthalmic Genetics, Casey Eye Institute, Oregon Health & Science University, and a Principal Investigator for the BRILLIANCE clinical trial commented, "I am proud to be able to offer patients potentially sight-restoring gene therapy treatments through clinical trials such as this, and am particularly proud to now be able to offer youth a chance to participate in the BRILLIANCE trial. If the trial's investigational treatment is found to be safe and effective, it could enable children born with mutations in the CEP290 gene an opportunity for a lifetime of sight."

The Company plans to present initial clinical data from the BRILLIANCE clinical trial at an upcoming medical meeting and is currently submitting an abstract to the International Symposium on Retinal Degeneration (RD2021) planned for September in Nashville.

### **About EDIT-101**

EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10). 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) 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. Additional details are available on [www.clinicaltrials.gov](http://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 two to 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, 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](http://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 expected first dosing of both the pediatric mid-dose cohort and the adult high-dose cohort in the BRILLIANCE trial this summer and completion of dosing of these cohorts in the first half of 2022,

and the Company's plan to report initial EDIT-101 clinical data at a medical meeting in September 2021. 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 because of new information, future events or otherwise.

Contacts:

Media

Cristi Barnett

(617) 401-0113

[cristi.barnett@editasmed.com](mailto:cristi.barnett@editasmed.com)

Investors

Ron Moldaver

(617) 401-9052

[ir@editasmed.com](mailto:ir@editasmed.com)



Source: Editas Medicine, Inc.