Editas Medicine Announces Positive Initial Clinical Data from Ongoing Phase 1/2 BRILLIANCE Clinical Trial of EDIT-101 for LCA10

September 29, 2021

No serious adverse events or dose-limiting toxicities observed to date

Efficacy signals in the mid-dose cohort provide initial support for clinical benefits

Treatment in the adult high-dose cohort continues and pediatric mid-dose cohort commencing

Data presented in an oral presentation at the International Symposium on Retinal Degeneration

Company to host a webcast today at 11:00 a.m. ET

CAMBRIDGE, Mass., Sept. 29, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced positive initial clinical data from the ongoing, open label Phase 1/2 BRILLIANCE clinical trial of EDIT-101. EDIT-101 is under development for the treatment of blindness due to Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder. The data, including preliminary patient safety and efficacy assessments relating to potential clinical benefits, are being presented today in an oral presentation at the XIXth International Symposium on Retinal Degeneration (RD2021) by Dr. Mark Pennesi, M.D., Ph.D.

"The preliminary results shared today support our belief that EDIT-101 has the potential to provide meaningful benefits to people living with *CEP290*-related retinal degeneration or LCA10. A positive safety profile has been observed through up to 15 months, with mostly mild adverse events primarily related to the procedure of retinal injection. The safety profile has allowed us to start enrolling and treating subjects in both the high-dose adult cohort and the mid-dose pediatric cohort. Early observations from individuals who were treated in the mid-dose cohort show clinical evidence that gene editing has occurred, demonstrated by visual improvements, as measured by full-field light sensitivity threshold (FST) testing, best corrected visual acuity (BCVA), or improvement in their ability to navigate standardized navigation courses with varying levels of difficulty. We will continue to follow the trial participants prospectively and collect clinical measures to allow us to determine the extent of both continued and durable improvements," said Lisa Michaels, M.D., Executive Vice President and Chief Medical Officer, Editas Medicine. "I would like to thank the participants, clinicians, and collaborating institutions that continue to contribute to this landmark gene editing medicine clinical trial."

"I am encouraged by these initial results, which indicate this investigational gene editing treatment has been well-tolerated in this trial's participants thus far and may also help improve sight for people with mutations in the *CEP290* gene. Being able to edit genes inside the human body is incredibly profound, and I hope to be able to offer my LCA patients new treatment options involving gene editing in the future," said Mark Pennesi, M.D., Ph.D., Professor of Molecular and Medical Genetics, Kenneth C. Swan Endowed Professor of Ophthalmology, Paul H. Casey Ophthalmic Genetics Division Chief, Casey Eye Institute, Oregon Health & Science University, and a BRILLIANCE principal investigator.

Dr. Michaels added, "These encouraging results provide a proof of concept on our *in vivo* gene editing platform and increase Editas' confidence in the broad potential of our gene editing technology to address additional serious diseases."

Preliminary Results

Preliminary results include safety and efficacy data from the first two cohorts, the adult low-dose cohort ($6x10^{11}$ vg/ml) and the adult mid-dose cohort ($1.1x10^{12}$ vg/ml). The BCVA eligibility criteria for the adult low-dose cohort (n=2) are light perception (LP), black-white discrimination, and white field projection. For the adult mid-dose and all other cohorts, the first subject is required to have light perception to BCVA of 1.6 logMAR (20/800 Snellen). BCVA criteria for all subsequent subjects is LP to 0.4 logMAR (20/50 Snellen).

Safety

Safety data was reported with respect to all six subjects treated in the low dose (n=2) and mid-dose (n=4) cohorts. Most adverse events (AEs) were mild and primarily resulting from the surgical procedure and subretinal injection. There were no dose limiting toxicities (DLTs) defined as a vision-threatening toxicity or severe non-ocular AE that occurs before or at the Week 4 visit and is assessed by the investigator as being related to EDIT-101 and not the administration procedure. Mild anterior chamber inflammation was observed, and adequately controlled with oral steroids. No Cas9-specific antibody or T-cell response was detected. To date, no treatment-related cataracts, edema, or retinal thinning have been observed.

Efficacy

Efficacy was assessed based on available data from five subjects treated in the adult low-dose cohort (n=2) and the adult mid-dose cohort (n=3), who had at least three months of post treatment follow-up, focusing on those measures demonstrated to be consistent and reproducible in subjects with *CEP290* retinal degeneration, including: BCVA, FST, and Visual Function Navigation (VNC™, developed by Ora, Inc.).

Two of three subjects in the mid-dose cohort followed for up to six months showed efficacy signals suggesting productive editing and providing initial support for clinical benefits, including improvements in BCVA, FST, and/or mobility navigation. BCVA is assessed by using the Early Treatment of Diabetic Retinopathy Study (ETDRS) eye chart, or the Berkeley Rudimentary Vision Test (BRVT), or the Lea Symbols 15-line pediatric eye chart. FST measures the sensitivity of the entire visual field by testing for the lowest luminance flash which provokes a visual sensation. Patients are presented with blue, red, and white stimuli, and based on the blue-red difference, the experimenter can determine the sensitivity of rod-mediated and cone-mediated perceptions. Navigational capabilities were assessed using standardized mobility testing with four different navigation courses, designed with varying levels of difficulty.

Mid-dose cohort Subject 1 showed improvement in BCVA of approximately 0.7 logMAR at Month 1.5 which was sustained at Month 6 follow-up.

In addition, there was a positive trend toward improved retinal sensitivity by FST in the study eye relative to the untreated eye. The subject also demonstrated a 5-level improvement in mobility at Month 6, as assessed with the VNC.

Mid-dose cohort Subject 2 showed improvement by Month 3 with a stable BCVA and a notable improvement in retinal sensitivity in the study eye relative to the untreated eye by FST, detectable at Month 1.5 that continued to improve through Month 3.

In June, the Independent Data Monitoring Committee (IDMC) endorsed proceeding with the first pediatric cohort based on a review of clinical safety data from the adult low-dose and adult mid-dose cohorts. Treatment in the adult high-dose cohort continues and the pediatric mid-dose cohort is commencing.

Investor Event and Webcast Information
Editas Medicine will host a live webcast today, Wednesday, September 29, 2021, at 11:00 a.m. ET to review the presented data. To join the webcast, please visit this **link** or visit the Events & Presentations page of the Investor section of the Company's website. A replay of the webcast will be available on the Editas Medicine website for 30 days following the call.

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), 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, such as the results presented in this press release, 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.

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