



## Editas Medicine Announces FDA Acceptance of IND Application for EDIT-101

November 30, 2018

*EDIT-101 set to be the first in vivo CRISPR medicine administered to people anywhere in the world*

*Earns \$25 million milestone for IND acceptance from partner Allergan*

CAMBRIDGE, Mass., Nov. 30, 2018 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ: EDIT), a leading genome editing company, today announced the U.S. Food and Drug Administration (FDA) has accepted the Company's Investigational New Drug (IND) application for EDIT-101, an experimental CRISPR genome editing medicine being investigated for the treatment of Leber Congenital Amaurosis type 10 (LCA10).

"The FDA's acceptance of our IND for EDIT-101 is a significant moment in the field of genome editing, and importantly, a critical milestone for patients, as we are now one step closer to a treatment for LCA10," said Katrine Bosley, President and Chief Executive Officer, Editas Medicine. "This moment is a truly exciting one for us, and we look forward to embarking on our next chapter as a clinical stage company, harnessing the power of CRISPR technology to transform the lives of people with serious diseases around the world."

With the IND acceptance, Editas Medicine has earned a \$25 million milestone payment from Allergan as part of the alliance between the companies to discover and develop experimental ocular medicines targeting serious, vision-threatening diseases. Editas Medicine and its partner, Allergan Pharmaceuticals International Limited (Allergan), a wholly-owned subsidiary of Allergan plc, expect to enroll 10 to 20 patients in a Phase 1/2 open label, dose escalation study to evaluate the safety, tolerability, and efficacy of EDIT-101.

### **About EDIT-101**

EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber Congenital Amaurosis type 10 (LCA10). EDIT-101 is administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells.

### **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 The Editas Medicine-Allergan Alliance**

In March 2017, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) entered a strategic alliance and option agreement under which Allergan received exclusive access and the option to license up to five of Editas Medicine's genome editing programs for ocular diseases, including EDIT-101. Under the terms of the agreement, Allergan is responsible for development and commercialization of optioned products, subject to Editas Medicine's option to co-develop and share equally in the profits and losses of two optioned products in the United States. In August 2018, Allergan exercised its option to develop and commercialize EDIT-101 globally for the treatment of LCA10. Additionally, Editas Medicine exercised its option to co-develop and share equally in the profits and losses from EDIT-101 in the United States. Editas Medicine is also eligible to receive development and commercial milestones, as well as royalty payments on a per-program basis. 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 (also known as Cas12a).

### **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/Cpf1 (also known as Cas12a) 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. 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 because of new information, future events or otherwise.

### **Contacts:**

#### **Media**

Cristi Barnett

(617) 401-0113

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

**Investors**

Mark Mullikin

(617) 401-9083

[mark.mullikin@editasmed.com](mailto:mark.mullikin@editasmed.com)



Source: Editas Medicine, Inc.