



## Editas Medicine Announces Third Quarter 2018 Results and Update

November 7, 2018

*Filed IND application for EDIT-101 with FDA in October*

*Favorable Appeals Court ruling affirms strength of foundational intellectual property*

*Oral presentation at ASH Annual Meeting to highlight novel Sickle Cell Disease and Beta-Thalassemia program*

*Strong balance sheet with \$337 million of cash, cash equivalents, and marketable securities as of September 30, 2018*

CAMBRIDGE, Mass., Nov. 07, 2018 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ: EDIT), a leading genome editing company, today reported business highlights and financial results for the third quarter ended September 30, 2018. In addition, the Company highlighted select corporate milestones and data from its pipeline of CRISPR medicines under investigation.

"The filing of the IND for EDIT-101 for the treatment of LCA10 marks a significant milestone for Editas and brings us closer to helping people living with this devastating disease," said Katrine Bosley, President and Chief Executive Officer of Editas Medicine. "We have also made important progress on our engineered cell medicines and we look forward to presenting data from our novel program to treat Sickle Cell Disease and Beta-Thalassemia in an oral presentation at the American Society of Hematology meeting next month."

### Recent Achievements and Outlook

- **Filed EDIT-101 Investigational New Drug (IND) application in October with U.S. Food and Drug Administration (FDA) for treatment of Leber Congenital Amaurosis type 10 (LCA10).** In addition, the National Institutes of Health (NIH) determined that a Recombinant DNA Advisory Committee review of the protocol for the Phase 1/2 trial of EDIT-101 was not necessary, and the protocol is now registered with the NIH. The Company expects to treat 10 to 20 patients in this open label, dose escalation study in order to evaluate the safety and efficacy of EDIT-101.
- **Allergan and Editas Medicine to co-develop and equally share profits and losses from EDIT-101 in the U.S.** In the third quarter, Allergan Pharmaceuticals International Limited (Allergan) exercised its option to develop and commercialize EDIT-101 under the strategic alliance formed in March 2017. Allergan paid an optionexercise fee of \$15 million, which was recorded in the third quarter. In addition, Editas Medicine is eligible to receive a \$25 million milestone payment from Allergan upon clearance of the IND application for EDIT-101. Editas Medicine also exercised its option in the third quarter to co-develop and equally share profits and losses in the U.S. for EDIT-101.
- **Novel approach to Sickle Cell Disease and Beta-Thalassemia to be highlighted in an oral presentation at the 60<sup>th</sup>American Society of Hematology (ASH) Annual Meeting.** Editas Medicine will present pre-clinical *in vivo* data comparing its novel gene editing strategy to reactivate fetal hemoglobin (HbF) by editing the beta-globin locus to strategies that edit the BCL11A erythroid enhancer (BCL11Ae). In this mouse study, editing the beta-globin locus upregulated HbF with superior repopulation of red blood cell precursors as compared to editing BCL11Ae.
- **Federal Appeals Court ruling affirms strength of foundational CRISPR/Cas9 intellectual property.** The U.S. Court of Appeals for the Federal Circuit (Appeals Court) upheld the U.S. Patent and Trademark Office no interference-in-fact decision concerning certain CRISPR/Cas9 patents Editas Medicine exclusively licenses from Broad Institute, Inc. The patents broadly cover CRISPR/Cas9 gene editing in eukaryotic cells, which the Company believes is essential to making CRISPR medicines.
- **Added key leadership in oncology and engineered cell medicines.** Richard A. Morgan, Ph.D., a leading expert in gene therapy and oncology, joined Editas Medicine as Senior Vice President of Immunogenetics. Dr. Morgan brings more than 30 years of scientific leadership in the life sciences industry.
- **Strong balance sheet to advance Editas Medicine through multiple value inflection points.** The Company held cash, cash equivalents, and marketable securities of \$337.5 million as of September 30, 2018, providing at least 24 months of funding for operating expenses and capital expenditures without any assumption of future cash received from milestones or additional financings.

### Upcoming Events

Editas Medicine will participate in the following investor conferences:

- Credit Suisse 27<sup>th</sup> Annual Healthcare Conference, November 13, 3:25 p.m. PT, Phoenix;
- Barclays Gene Editing & Gene Therapy Summit, November 29, 1:15 p.m. ET, New York City; and
- J.P. Morgan 37<sup>th</sup> Annual Healthcare Conference, January 7-10, 2019, San Francisco

Editas Medicine will present pre-clinical data from its novel program to address Sickle Cell Disease and Beta-Thalassemia in an oral presentation at the 60<sup>th</sup> ASH Annual Meeting. Details are as follows:

Oral Abstract Number: 409

Title: *Comparative Studies Reveal Robust HbF Induction By Editing of HBG1/2 Promoters or BCL11A Erythroid-Enhancer in Human CD34+ Cells but That BCL11A Erythroid-Enhancer Editing Is Associated with Selective Reduction in Erythroid Lineage Reconstitution in a Xenotransplantation Model*

Presenter: KaiHsin Chang, Ph.D.

Session: 112. Thalassemia and Globin Gene Regulation: Hemoglobin Switching

Time: Sunday, December 2, 4:30 p.m.

Location: San Diego Convention Center, Room 25B

### Third Quarter 2018 Financial Results

Cash, cash equivalents, and marketable securities at September 30, 2018, were \$337.5 million, compared to \$329.1 million at December 31, 2017.

For the third quarter ended September 30, 2018, net loss attributable to common stockholders was \$15.2 million, or \$0.32 per share, compared to \$26.6 million, or \$0.64 per share, for the same period in 2017.

- Collaboration and other research and development revenues were \$14.5 million for the quarter ended September 30, 2018, compared to \$6.3 million for the same period in 2017. The \$8.2 million increase was primarily attributable to a \$10.6 million increase in revenue recognized pursuant to our strategic alliance with Allergan and \$0.1 million in revenue recognized pursuant to a license agreement with Beam Therapeutics, Inc., partially offset by a \$2.4 million decrease in revenue recognized pursuant to our collaboration agreement with Juno Therapeutics, Inc., a Celgene company and wholly-owned subsidiary of Celgene Corporation.
- Research and development expenses were \$17.4 million for the quarter ended September 30, 2018, compared to \$20.4 million for the same period in 2017. The \$3.0 million decrease was primarily attributable to \$4.9 million in decreased sublicensing expenses and \$1.0 million in decreased process and platform development expenses, mostly related to reimbursable expenses related to the Allergan profit-sharing arrangement. These decreases were partially offset by \$1.3 million in increased employee related expenses, \$1.1 million in increased stock-based compensation expenses, \$0.3 million in increased facility-related expenses and \$0.3 million in increased other expenses.
- General and administrative expenses were \$13.3 million for the quarter ended September 30, 2018, compared to \$12.6 million for the same period in 2017. The \$0.7 million increase was primarily attributable to \$1.2 million in increased stock-based compensation expenses, \$1.1 million in increased employee related expenses, \$0.7 million in increased professional service expenses and \$0.2 million in increased other expenses, partially offset by \$2.4 million in decreased intellectual property and patent related fees.

### Conference Call

The Editas Medicine management team will host a conference call and webcast today, November 7, 2018, at 5:00pm ET. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 8397886. A live webcast of the call will be available on the Investors & Media section of the Editas Medicine website at [www.editasmedicine.com](http://www.editasmedicine.com) and a replay will be available approximately two hours after its completion.

### 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).

### 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 an AAV5 construct and 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 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. Editas Medicine is also eligible to receive development and commercial milestones, as well as royalty payments on a per-program basis where the parties are not sharing profits and losses. 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).

#### 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 "aim," "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 Company planning to present data and the Company developing and bringing transformative medicines to patients. 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 as a result of new information, future events or otherwise.

**Editas Medicine, Inc.**  
**Condensed Consolidated Statements of Operations**  
**(unaudited)**  
**(amounts in thousands, except per share and share data)**

	<b>Three Months Ended</b>	
	<b>September 30,</b>	
	<b>2018</b>	<b>2017</b>
Collaboration and other research and development revenues	\$ 14,519	\$ 6,282
Operating expenses:		
Research and development	17,443	20,396
General and administrative	13,334	12,635
Total operating expenses	30,777	33,031
Operating loss	(16,258)	(26,749)
Other income, net:		
Other (expense) income, net	(4)	196
Interest income (expense), net	1,024	(46)
Total other income, net	1,020	150
Net loss	\$ (15,238)	\$ (26,599)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.32)	\$ (0.64)
Weighted-average common shares outstanding, basic and diluted	47,414,271	41,307,092

**Editas Medicine, Inc.**  
**Selected Condensed Consolidated Balance Sheet Items**  
**(unaudited)**  
**(amounts in thousands)**

<b>September 30,</b>	<b>December 31,</b>
<b>2018</b>	<b>2017</b>

Cash, cash equivalents, and marketable securities	\$	337,492	\$	329,139
Working capital		316,467		295,492
Total assets		387,630		373,260
Deferred revenue, net of current portion		104,100		94,725
Construction financing lease obligation, net of current portion		32,694		33,431
Total stockholders' equity		224,449		208,080

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