

## Editas Medicine Announces Fourth Quarter and Full Year 2016 Results and Update

March 7, 2017 4:03 PM ET

*Recent, highly favorable U.S. Patent and Trademark Office decision affirms and upholds fundamental CRISPR/Cas9 issued patents*

*Extended CRISPR technology leadership with addition of novel and differentiated Cpf1 system, advanced forms of Cas9*

*Achieved in vivo proof-of-editing of non-human primate retina in LCA10 program*

CAMBRIDGE, Mass., March 07, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today reported financial results for the fourth quarter and full year 2016. The Company also provided an overview of 2016 achievements and 2017 goals.

“In three years, we have established an unparalleled position for developing genome editing medicines,” said Katrine Bosley, President and Chief Executive Officer of Editas Medicine. “In 2016, we significantly broadened our scientific platform with CRISPR/Cpf1 and advanced forms of CRISPR/Cas9, demonstrated proof-of-editing *in vivo* for our most advanced program in LCA10, and expanded our leadership team with the addition of our Chief Scientific Officer and Chief Medical Officer.”

Bosley added, “2017 is already shaping up to be a transformative year for Editas. The recent, highly favorable decision by the U.S. Patent & Trademark Office affirms and upholds the broad and fundamental patents in this field granted to the Broad Institute and exclusively licensed to Editas for human therapeutics. We believe these patents will apply to all CRISPR/Cas9-based medicines.”

### Key Achievements

*Driving Editas Medicine’s unparalleled platform for genome editing medicines*

- **Reinforced robust Cas9 intellectual property.** Highly favorable decision by US Patent and Trademark Office (USPTO) in February 2017 affirmed and upheld the Broad Institute, Inc.’s (Broad) fundamental CRISPR/Cas9 patents and reinforces Editas Medicine’s unmatched intellectual property portfolio for CRISPR medicines. The USPTO determined that Broad’s invention using CRISPR/Cas9 in eukaryotic cells was not obvious in view of University of California, University of Vienna, and Emmanuelle Charpentier’s claims in their patent application and thus ended the interference and upheld the Broad issued patents. Foundational claims covering the use of CRISPR/Cas9 for gene editing in eukaryotic cells have issued to the Broad as patents in each of the United States, Europe, and Australia.
- **Expanded unparalleled and differentiated platform for genome editing medicines with exclusive licenses for the CRISPR/Cpf1 system and for advanced forms of Cas9.** Editas Medicine’s platform now includes exclusive rights to both CRISPR systems that have been demonstrated to work in human cells: Cas9 and Cpf1. Cpf1 offers several potential advantages including expanding the range of genomic sites that can be edited, simplifying manufacture and delivery, and increased efficiency and accuracy of some forms of gene repair. Its intellectual property rights are independent of Cas9.
- **Invented and deployed new proprietary technologies** to potentially enable unprecedented medicines. Editas Medicine achieved multiple technical advancements including novel compositions and methods for covalently-coupled dual guide RNAs and rigorous new empirical methods for assessing specificity referred to as Uni-directional Targeted Sequencing (UDITAS™).

*Advancing a pipeline strategy to enable successful product development in the years ahead*

- **Achieved *in vivo* proof-of-editing in non-human primates** in our Leber Congenital Amaurosis type 10 (LCA10) program. Our advancements in the LCA10 program establish the foundation for developing a wide range of potential treatments for inherited retinal diseases and diseases of the eye, as well as other systemic therapies.
- **Demonstrated efficient editing of hematopoietic stem cells (HSCs) and long-term engraftment and reconstitution of the blood and bone marrow** using edited cells in animal models. Editas Medicine's progress in HSCs provides the foundation for potential CRISPR medicines to treat a broad range of diseases of the blood and bone marrow, including sickle cell disease and immunological diseases.
- **Attained over 90% knock-out of PD-1 in T cells carrying a chimeric antigen receptor (CAR) with no detected off-target edits** in our collaboration with Juno Therapeutics, Inc. (Juno Therapeutics). In our wholly-owned T cell research outside of cancer, we attained similar high levels of editing and targeted integration that creates the potential to treat a broad array of immunological and infectious diseases.

*Building the business for the long term by assembling the capabilities to fully develop and commercialize important medicines*

- **Successfully completed initial public offering** resulting in \$109 million in gross proceeds including full exercise of underwriters' over-allotment option.
- **Expanded and strengthened executive team** with multiple appointments in 2016 and early 2017, including:
  - o Charles Albright, Ph.D., Chief Scientific Officer;
  - o Gerald Cox, M.D., Ph.D., Chief Medical Officer;
  - o Timothy Hunt, J.D., Senior Vice President of Corporate Affairs;
  - o Haiyan Jiang, Ph.D., Vice President of Preclinical Science;
  - o Pamela Stetkiewicz, Ph.D., Vice President of Program and Alliance Management;
  - o Kenneth LeClair, Ph.D., Vice President of Technical Development and Manufacturing;
  - o Semiramis Trotto, Vice President of Human Resources; and
  - o Kevin Dushney, Vice President of Information Technology.
- **Established important alliances to further extend platform leadership and advance our pipeline.** We are working with Adverum Biotechnologies to explore the use of Adverum's next-generation adeno-associated virus (AAV) vectors in up to five ophthalmic indications. Through our collaboration with San Raffaele Telethon Institute for Gene Therapy (TIGET), one of the world's leading institutes for genomic medicines, we aim to advance our pipeline of HSC and T cell therapies.

## 2017 Goals

Editas Medicine has established the following goals for 2017:

- Submit IND for LCA10 program by end of year;
- Initiate LCA10 clinical natural history study mid-year;
- Achieve pre-clinical proof-of-concept for additional programs;
- Establish additional alliances to enable successful product development; and
- Continue to build outstanding organization and culture.

## Upcoming Events

Editas Medicine will participate in the following upcoming investor conferences:

- Barclays Global Healthcare Conference, March 16, 9:30 a.m. ET, Miami; and

- UBS Global Healthcare Conference, May 22-24, New York City.

Editas Medicine will participate in the following upcoming scientific and medical conferences:

- Keystone Symposium on Genomic Instability and DNA Repair, April 2-6, Santa Fe;
- Tides 2017: Oligonucleotide and Peptide Therapeutics, April 30-May 3, San Diego; and
- American Society of Gene & Cell Therapy (ASGCT), May 10-13, Washington, DC.

#### **Fourth Quarter and Full Year 2016 Financial Results**

Cash and cash equivalents at December 31, 2016, were \$185.3 million, compared to \$199.9 million at September 30, 2016, and \$143.2 million at December 31, 2015.

For the three months ended December 31, 2016, net loss attributable to common stockholders was \$39.4 million, or \$1.10 per share, compared to \$12.7 million, or \$4.05 per share, for the same period in 2015.

- Collaboration and other research and development revenues were \$0.9 million for the three months ended December 31, 2016, compared to \$0.8 million for the same period in 2015. The \$0.1 million increase was due to an increase in revenue recognized pursuant to our collaboration with Cystic Fibrosis Foundation Therapeutics, Inc.
- Research and development expenses increased by \$21.0 million, to \$26.8 million for the three months ended December 31, 2016, from \$5.8 million for the same period in 2015. The \$21.0 million increase was due to a \$16.5 million increase in license fees, primarily related to new license agreements with Massachusetts General Hospital and the Broad Institute, a \$2.1 million increase in employee and non-employee related expenses, including stock-based compensation, resulting from an increase in the size of our workforce, a \$1.2 million increase in our process and platform development expenses due to increased research activity, and a \$1.2 million increase in facility-related costs resulting from additional office and laboratory space.
- General and administrative expenses increased by \$5.7 million to \$13.0 million for the three months ended December 31, 2016, from \$7.3 million for the same period in 2015. The \$5.7 million increase in general and administrative expenses consisted of an increase of \$3.5 million in legal fees to support patents that we own or in-license, including costs for the prosecution and maintenance of patents that we own or in-license as well as to procure the application for and issuance of additional patents in the United States and other jurisdictions, an increase of \$1.6 million in employee compensation costs, and a \$0.6 million increase in other general and administrative expenses including office and facility costs related to our new headquarters.

For the full year 2016, net loss attributable to common stockholders was \$97.2 million, or \$3.02 per share, compared to \$73.3 million, or \$28.55 per share, for 2015.

- Collaboration and other research and development revenues were \$6.1 million for 2016, compared to \$1.6 million for 2015. The increase of \$4.5 million was due to a \$4.2 million increase in revenue recognized pursuant to our collaboration with Juno Therapeutics, and a \$0.3 million increase in revenue recognized pursuant to our agreement with Cystic Fibrosis Foundation Therapeutics.
- Research and development expenses for 2016 were \$57.0 million, compared to \$18.8 million for 2015. The increase of \$38.1 million was due to a \$14.6 million increase in research and development employee and non-employee compensation costs, including stock based compensation, a \$13.1 million increase in license fees primarily related to new license agreements with Massachusetts General Hospital and the Broad Institute, \$6.4 million in increased process and platform development costs, \$3.9 million in increased facilities costs, and \$0.1 million in increased other expenses.
- General and administrative expenses were \$46.3 million for 2016, compared to \$18.1 million for 2015. The increase

of \$28.2 million was due to a \$16.5 million increase in legal fees to support patents that we own or in-license, including costs for the prosecution and maintenance of patents that we own or in-license, \$7.2 million in increased employee compensation costs, \$2.4 million in increased contractor consulting fees, and \$2.1 million in other general and administrative expenses.

- Other income (expense), net for 2016 was \$5 thousand, compared to \$(37.6) million for 2015. The decrease in expense was primarily related to a \$35.6 million decrease in our Series A preferred stock tranche right liability, which was settled in June 2015, resulting from mark-to-market adjustments attributable to an increase in the fair value of our Series A preferred stock during 2015, and to a \$1.6 million mark-to-market adjustment recorded in June 2015 for the anti-dilution protection liability, which was also settled in June 2015, related to our issuance of common stock to our licensors.

## **Conference Call**

The Editas Medicine management team will host a conference call and webcast today at 5:00 p.m. ET to provide and discuss a corporate update and financial results for the fourth quarter and full year 2016. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 81030032. 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**

Editas Medicine is a leading genome editing company dedicated to treating patients with genetically-defined diseases by correcting their disease-causing genes. The Company was founded by world leaders in genome editing, and its mission is to translate the promise of genome editing science into a broad class of transformative genomic medicines to benefit the greatest number of patients.

## **Forward-Looking Statements**

This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995, including statements regarding the Company's goals of submitting of an IND for the LCA10 program by the end of 2017, initiating an LCA10 clinical natural history study in mid-2017, achieving preclinical proof-of-concept for additional programs, and establishing additional alliances to enable successful product development. 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 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 as a result of new information, future events or otherwise.

**EDITAS MEDICINE, INC.****Consolidated Statement of Operations**

(amounts in thousands, except share and per share data)

(Unaudited)

	<b>Three Months Ended December 31,</b>		<b>Twelve Months Ended December 31,</b>	
	<b>2016</b>	<b>2015</b>	<b>2016</b>	<b>2015</b>
Collaboration and other research and development revenues	\$ 898	\$ 792	\$ 6,053	\$ 1,629
Operating expenses:				
Research and development	26,835	5,826	56,979	18,846
General and administrative	13,047	7,339	46,262	18,095
Total operating expenses	39,882	13,165	103,241	36,941
Operating loss	(38,984 )	(12,373 )	(97,188 )	(35,312 )
Other income (expense), net:				
Other expense, net	(35 )	(226 )	(57 )	(37,445 )
Interest income (expense), net	(357 )	(34 )	62	(143 )
Total other income (expense), net	(392 )	(260 )	5	(37,588 )
Net loss and comprehensive loss	\$ (39,376 )	\$ (12,633 )	\$ (97,183 )	\$ (72,900 )
Accretion of redeemable convertible preferred stock to redemption value	\$ -	\$ (99 )	\$ (47 )	\$ (394 )
Net loss attributable to common stockholders	\$ (39,376 )	\$ (12,732 )	\$ (97,230 )	\$ (73,294 )
Net loss per share attributable to common stockholders, basic and diluted	\$ (1.10 )	\$ (4.05 )	\$ (3.02 )	\$ (28.55 )
Weighted-average common shares outstanding, basic and diluted	35,731,230	3,145,380	32,219,717	2,566,916

**EDITAS MEDICINE, INC.****Selected Consolidated Balance Sheet Items**

(amounts in thousands)

(Unaudited)

	<b>December 31, 2016</b>	<b>December 31, 2015</b>
Cash and cash equivalents	\$ 185,323	\$ 143,180
Working capital	154,100	138,060
Total assets	229,182	149,363

Deferred revenue, net of current	26,000	25,321
Construction financing lease obligation, net of current portion	35,096	-
Redeemable convertible preferred stock	-	199,915
Total stockholders' equity (deficit)	134,607	(83,114 )

Media Contact

Dan Budwick

Pure Communications, Inc.

(973) 271-6085

[dan@purecommunicationsinc.com](mailto:dan@purecommunicationsinc.com)

Investor Contact

Mark Mullikin

Editas Medicine, Inc.

(617) 401-9083

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



Editas Medicine