



Editas Medicine Announces Fourth Quarter and Full Year 2018 Results and Update

February 28, 2019

EDIT-101 for LCA10 set to be first ever in vivo CRISPR medicine administered to patients with dosing expected in second half of 2019

Advancing potentially best-in-class engineered cell medicine for sickle cell disease and beta-thalassemia

Appointed Cynthia Collins and David Scadden, M.D., to Board of Directors and named Collins as interim Chief Executive Officer

Year-end cash, cash equivalents, and marketable securities of \$369 million expected to fund advancement of multiple transformative experimental medicines

CAMBRIDGE, Mass., Feb. 28, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the fourth quarter and full year 2018.

"2018 was a year of significant achievements for Editas Medicine as we advanced our efforts to bring transformative medicines to patients," said Cynthia Collins, interim Chief Executive Officer, Editas Medicine. "The IND application for our lead experimental medicine, EDIT-101 for LCA10, was accepted by the FDA upon initial review. We advanced a potentially best-in-class CRISPR medicine candidate to treat sickle cell disease and beta-thalassemia; and, we strengthened our organization with key additions to our senior leadership and Board of Directors. In 2019, we plan to dose patients with EDIT-101, making it the first *in vivo* CRISPR medicine administered to patients in history. We are also well positioned to advance the Company's EM22 long-range goals."

Recent Achievements and Outlook

- **EDIT-101 Investigational New Drug (IND) application accepted upon initial review with patient dosing planned for second half of 2019.** The U.S. Food and Drug Administration accepted the IND application in November 2018 following its initial 30-day review. As a result of the IND acceptance, Editas Medicine received a \$25 million milestone payment from Allergan Pharmaceuticals International Limited (Allergan) as part of the alliance between the companies to discover and develop ocular medicines targeting serious, vision-threatening diseases. Editas Medicine and Allergan expect to enroll 10-20 patients in a Phase 1/2 open label, dose escalation study to evaluate the safety, tolerability, and efficacy of EDIT-101 as a treatment for Leber congenital amaurosis 10 (LCA10). EDIT-101 is expected to be the first *in vivo*, or editing inside the body, CRISPR medicine administered to patients in history.
- **Advancing research program to address Usher syndrome 2A (USH2A).** The Company and collaborators from Massachusetts Eye and Ear plan to present *in vivo* proof-of-concept data for a CRISPR gene editing approach for USH2A at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy.
- **Potential for best-in-class engineered cell medicine for sickle cell disease and beta-thalassemia.** Editas Medicine expects to begin IND-enabling activities for an experimental medicine candidate for sickle cell disease and beta-thalassemia in 2019. At the 60th Annual Meeting of the American Society of Hematology, the Company presented data on its differentiated approach to developing a durable genomic medicine for sickle cell disease and beta-thalassemia. In these pre-clinical mouse data, Editas Medicine showed that editing the beta-globin locus upregulated fetal hemoglobin with superior repopulation of red blood cell precursors as compared to editing the BCL11A erythroid enhancer.
- **Expanding investment in engineered cell medicines for cancer.** In addition to the Company's collaboration with Juno Therapeutics, a Celgene company and wholly-owned subsidiary of Celgene Corporation (Juno Therapeutics), to develop engineered T cell medicines to treat cancer, Editas Medicine is also advancing wholly-owned research programs to develop allogeneic engineered natural killer cell medicines in oncology.
- **Preparing organization for the next phase of growth.** Cynthia Collins, a recognized leader with more than 30 years of experience in cell and gene therapy, molecular diagnostics, life sciences tools, and therapeutics, was appointed to the Board of Directors (Board) in December 2018 and named interim Chief Executive Officer in January 2019. The Board has retained a leading executive search firm to assist in identifying a permanent successor. In addition, David Scadden, M.D., professor of medicine at Harvard University and director of the Center for Regenerative Medicine at Massachusetts General Hospital, was appointed to the Board in February 2019.
- **Strong balance sheet to fund business through multiple important milestones.** The Company held cash, cash

equivalents, and marketable securities of \$369 million as of December 31, 2018, providing at least 24 months of funding for operating expenses and capital expenditures.

Upcoming Events

Editas Medicine will participate in the following investor events:

- Cowen & Company 39th Annual Health Care Conference, March 11, 4:50 p.m. ET, Boston;
- Barclays Global Healthcare Conference, March 14, 11:15 a.m. ET, Miami

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

- 6th Annual Retinal Cell and Gene Therapy Innovation Summit, April 26, Vancouver;
- 22nd Annual Meeting of the American Society of Gene & Cell Therapy, April 29-May 2, Washington, DC.

Fourth Quarter and Full Year 2018 Financial Results

Cash, cash equivalents, and marketable securities at December 31, 2018, were \$369.0 million, compared to \$337.5 million at September 30, 2018, and \$329.1 million at December 31, 2017.

For the three months ended December 31, 2018, net loss attributable to common stockholders was \$25.1 million, or \$0.52 per share, compared to \$36.2 million, or \$0.84 per share, for the same period in 2017.

- Collaboration and other research and development revenues were \$6.1 million for the three months ended December 31, 2018, compared to \$3.7 million for the same period in 2017. The \$2.4 million increase was primarily due to \$1.6 million in increased revenue recognized pursuant to our strategic alliance with Allergan and \$0.8 million in increased revenue recognized pursuant to our collaboration agreement with Juno Therapeutics.
- Research and development expenses decreased by \$7.2 million, to \$19.2 million for the three months ended December 31, 2018, from \$26.4 million for the same period in 2017. The \$7.2 million decrease was related to \$9.5 million in decreased success payment expenses, \$2.7 million in decreased stock-based compensation expenses and \$0.9 million in decreased process and platform development expenses, partially offset by \$3.8 million in increased sublicense payment expenses, \$1.3 million in increased employee related expenses and \$0.8 million in increased other expenses including facility-related expenses.
- General and administrative expenses decreased by \$0.5 million to \$13.2 million for the three months ended December 31, 2018, from \$13.7 million for the same period in 2017. The \$0.5 million decrease was related to \$2.5 million in decreased intellectual property and patent related fees, partially offset by \$0.9 million in increased stock-based compensation expenses, \$0.6 million in increased employee related expenses and \$0.4 million in increased other expenses including facility-related expenses and professional service expenses.

For the full year 2018, net loss attributable to common stockholders was \$110.0 million, or \$2.33 per share, compared to \$120.3 million, or \$2.98 per share, for the same period in 2017.

- Collaboration and other research and development revenues were \$31.9 million for 2018, compared to \$13.7 million for 2017. The increase of \$18.2 million was primarily due to \$12.7 million in increased revenue recognized related to our strategic alliance with Allergan, \$4.0 million in revenue recognized pursuant to a license agreement with Beam Therapeutics, Inc. and \$1.5 million in increased revenue recognized pursuant to our collaboration agreement with Juno Therapeutics.
- Research and development expenses were \$90.7 million for 2018, compared to \$83.2 million for 2017. The increase of \$7.5 million was due to \$8.3 million in increased process and platform development expenses, \$5.4 million in increased employee related expenses and \$2.0 million in increased other expenses including facility-related expenses, partially offset by \$5.9 million in decreased licensing and sublicensing payment expenses, \$2.0 million in decreased success payment expenses and \$0.4 million in decreased stock-based compensation expenses.
- General and administrative expenses were \$55.0 million for 2018, compared to \$50.5 million for 2017. The increase of \$4.5 million was due to \$3.6 million in increased stock-based compensation expenses, \$2.6 million in increased employee related expenses, \$0.9 million in increased other expenses including facility-related expenses and \$0.9 million in increased professional service expenses, partially offset by \$3.5 million in decreased intellectual property and patent related fees.

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 2018. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 4134877. A live webcast of the call will be available on the Investors & Media section of the Editas Medicine website at

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.

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 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).

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 Company's EM22 goals and plans to dose patients with EDIT-101 in the second half of 2019. 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.

EDITAS MEDICINE, INC.

Consolidated Statement of Operations

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

(Unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	2017	2018	2017
Collaboration and other research and development revenues	\$ 6,119	\$ 3,667	\$ 31,937	\$ 13,728
Operating expenses:				
Research and development	19,195	26,424	90,654	83,159
General and administrative	13,177	13,685	55,010	50,502
Total operating expenses	32,372	40,109	145,664	133,661
Operating loss	(26,253)	(36,442)	(113,727)	(119,933)
Other income (expense), net:				
Other (expense) income, net	(3)	129	328	587
Interest income (expense), net	1,202	124	3,445	(978)
Total other income (expense), net	1,199	253	3,773	(391)

Net loss	\$ (25,054)	\$ (36,189)	\$ (109,954)	\$ (120,324)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.52)	\$ (0.84)	\$ (2.33)	\$ (2.98)
Weighted-average common shares outstanding, basic and diluted	48,006,980	42,593,917	47,097,735	40,323,631

EDITAS MEDICINE, INC.
Selected Consolidated Balance Sheet Items
(amounts in thousands)
(Unaudited)

	<u>December 31, 2018</u>	<u>December 31, 2017</u>
Cash, cash equivalents, and marketable securities	\$ 368,955	\$ 329,139
Working capital	338,876	295,492
Total assets	420,386	373,260
Deferred revenue, net of current portion	115,614	94,725
Construction financing lease obligation, net of current portion	32,417	33,431
Total stockholders' equity	236,162	208,080

Investor Contact

Mark Mullikin
(617) 401-9083
mark.mullikin@editasmed.com

Media Contact

Cristi Barnett
(617) 401-0113
cristi.barnett@editasmed.com



Source: Editas Medicine, Inc.