Editas Medicine Announces Third Quarter 2017 Results and Update

November 7, 2017

CAMBRIDGE, Mass., Nov. 07, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today reported financial results for the third quarter ended September 30, 2017, and provided an update on recent achievements and upcoming events.

"This was a quarter of continued steady progress in advancing our pipeline of CRISPR medicines toward the clinic," said Katrine Bosley, President and Chief Executive Officer of Editas Medicine. "We were pleased to announce that we initiated our clinical natural history study for our lead program to treat LCA10, and we remain on track to file our IND for EDIT-101, our clinical candidate, in mid-2018. In addition, we look forward to data that will be presented at the upcoming meetings of the Society for Immunotherapy of Cancer and American Society of Hematology from a range of our wholly-owned and partnered engineered cell therapy programs."

Recent Achievements

Advancing product candidates and platform to deliver a sustainable pipeline of transformative products

EDIT-101 for Leber Congenital Amaurosis type 10 (LCA10) remains on track for a mid-2018 IND filing

- **Demonstrated dose-dependent editing with EDIT-101 that was durable through six months in a pre-clinical, transgenic mouse model.** In a study of transgenic mice carrying a copy of the mutated human gene (*CEP290*) targeted by our clinical candidate, EDIT-101, we demonstrated efficient transduction and gene editing in the retinal photoreceptor cells, the relevant cell type affected in LCA10 patients. Significant *CEP290* editing was rapidly achieved and sustained through six months of observation. In addition, both Cas9 messenger RNA and guide RNA levels correlated with editing. These data were presented recently at the European Society of Gene and Cell Therapy congress and further support advancing EDIT-101 towards clinical development.

- **Initiated clinical natural history study to facilitate interventional trial design and enrollment.** This study will prospectively evaluate patients with LCA10 across a broad range of ages and disease severity to assess the course of the disease and to pilot potential clinical trial endpoints and designs. The results of the study will inform interventional clinical trial design and enrollment for EDIT-101. Editas Medicine plans to enroll approximately 40 patients in this study, ages three and above, at multiple sites in the U.S. and Europe. Patients will be evaluated and followed for at least one year. Massachusetts Eye and Ear Institute, an international center for treatment and research and a teaching hospital of Harvard Medical School, is the first site enrolling patients for this study.

- **Received Orphan Medicinal Product Designation from the European Medicines Agency for EDIT-101.** Orphan Medicinal Product Designation is intended to support the development of medicines in debilitating disease indications with low prevalence and no satisfactory existing therapies.

Made important progress in engineered cell therapy programs

- **Established compelling pre-clinical data for two additional gene targets in our collaboration with Juno Therapeutics, Inc. (Juno Therapeutics), to develop next-generation engineered T cells for cancer.** Improving the ability of T cells to overcome the tumor microenvironment may expand the range of cancers that can be addressed by engineered T cells. In data to be presented by Juno Therapeutics and Editas Medicine scientists at the 32nd Annual Meeting of the Society for Immunotherapy of Cancer, T cells edited to knock out the *CBLB* gene will be shown to more efficiently kill antigen presenting target tumors cells, have enhanced cytokine production, and have greater proliferative capacity and survival rates in response to antigen stimulation, relative to unedited controls. In data to be presented at the 59th American Society of Hematology (ASH) Annual Meeting, Juno Therapeutics and Editas Medicine scientists will report knockout of the *TGFBR2* gene using CRISPR gene editing in BCMA-specific CAR-T cells for multiple myeloma prevented the development of the TGF beta-induced gene expression phenotype.

- **Advanced exploration of a potentially superior therapy for sickle cell disease and beta thalassemia.** In data to be presented at ASH, we will demonstrate high levels of gene disruption of adult human hematopoietic stem cells with CRISPR/Cpf1 and efficient targeted integration at the beta-hemoglobin locus with CRISPR/Cas9. We believe our data demonstrates multiple opportunities to develop best-in-class therapies for hemoglobinopathies.

Developing an outstanding organization

- **Expanded capabilities that we believe will be critical to our long-term success.** We added key talent in regulatory

affairs, manufacturing, and ophthalmology research. This expertise is critical to the continued advancement of our platform and pipeline.

Upcoming Events

Editas Medicine will participate in the following upcoming investor conferences:

- Barclays Gene Editing & Gene Therapy Summit, November 30, New York City; and
- Oppenheimer Rare/Orphan Disease Day, December 5, New York City.

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

- 32nd Annual Meeting of the Society for Immunotherapy of Cancer (SITC), November 8-12, National Harbor, MD; and
- 59th American Society of Hematology Annual Meeting (ASH), December 9-12, Atlanta, GA.

Third Quarter 2017 Financial Results

Cash, cash equivalents, and marketable securities at September 30, 2017, were $295.7 million, compared to $199.9 million at September 30, 2016.

For the third quarter ended September 30, 2017, net loss attributable to common stockholders was $26.6 million, or $0.64 per share, compared to $21.0 million, or $0.59 per share, for the same period in 2016.

- Collaboration and other research and development revenues were $6.3 million for the quarter ended September 30, 2017, compared to $1.0 million for the same period in 2016. The $5.3 million increase was primarily attributable to a $3.2 million increase in revenue recognized pursuant to our strategic alliance with Allergan and a $2.5 million milestone recognized pursuant to our collaboration with Juno Therapeutics, Inc., partially offset by a $0.4 million decrease in reimbursable research and development expenses.

- Research and development expenses were $20.4 million for the quarter ended September 30, 2017, compared to $10.8 million for the same period in 2016. The $9.6 million increase was primarily attributable to a $7.2 million increase in sublicensing payment expenses, a $1.6 million increase in process and platform development costs, and a $1.5 million increase in employee related expenses. This increase was partially offset by a $0.3 million decrease in facility related costs, a $0.3 million decrease in other expenses, and a $0.2 million decrease in stock-based compensation expenses.

- General and administrative expenses were $12.6 million for the quarter ended September 30, 2017, compared to $11.3 million for the same period in 2016. The $1.3 million increase was primarily attributable to a $1.0 million increase in stock-based compensation expenses, a $0.3 million increase in intellectual property legal and patent-related fees, and a $0.2 million increase in employee related expenses. This increase was partially offset by a $0.2 million decrease in other expenses.

Conference Call

The Editas Medicine management team will host a conference call and webcast today, November 7, 2017, at 5:00pm ET. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 9487629. 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

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. 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 goal of submitting an IND for EDIT-101 by mid-2018. 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)

	Three Months Ended September 30,	
	2017	**2016**
Collaboration and other research and development revenues	$ 6,282	$ 962
Operating expenses:		
Research and development	20,396	10,832
General and administrative	12,635	11,295
Total operating expenses	33,031	22,127
Operating loss	(26,749)	(21,165)
Other income, net:		
Other income, net	196	3
Interest income (expense), net	(46)	142
Total other income, net	150	145
Net loss	$ (26,599)	$ (21,020)
Net loss attributable to common stockholders	$ (26,599)	$ (21,020)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.64)	$ (0.59)
Weighted-average common shares outstanding, basic and diluted	41,307,092	35,505,429

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

	September 30, 2017	**December 31, 2016**
Cash, cash equivalents, and marketable securities	$ 295,691	$ 185,323
Working capital	268,920	154,100
Total assets	339,127	229,182
Deferred revenue, net of current portion	97,851	26,000
Construction financing lease obligation, net of current portion	33,667	35,096
Total stockholders' equity	177,788	134,607

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