Editas Medicine Announces Third Quarter 2020 Results and Update

November 5, 2020

EDIT-101 for LCA10 – BRILLIANCE trial adult low-dose cohort completed

EDIT-301 for sickle cell disease – on track for IND filing by end of 2020

EDIT-201 for solid tumors – preclinical data to be presented at SITC and ASH

Cash, cash equivalents, and marketable securities of $541 million as of September 30, 2020

CAMBRIDGE, Mass., Nov. 05, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the third quarter of 2020.

"We were pleased to regain full operating control of our ocular programs per our new agreement with AbbVie, which provides us with important flexibility. We have finished dosing the first cohort with EDIT-101 in the landmark BRILLIANCE trial and enrollment remains active," said Cynthia Collins, Chief Executive Officer of Editas Medicine.

Ms. Collins continued, "The advancements in our ocular portfolio are complemented by the strategic development of our engineered cell medicines. We are on track to file the IND for EDIT-301 for sickle cell disease in the fourth quarter and are eager to present additional data on this potentially best-in-class medicine and our large-scale manufacturing process at the upcoming American Society of Hematology Annual Meeting. Substantial progress has also been made in the development of EDIT-201, an allogeneic NK cell medicine to treat solid tumors. Preclinical data to be presented at ASH show enhanced tumor killing with EDIT-201 compared to unedited NK cells, and additional data on this program will be featured at the upcoming Society for Immunotherapy of Cancer's Annual Meeting."

Recent Achievements and Outlook

***In Vivo* CRISPR Medicines**

- **EDIT-101 for LCA10**
  BRILLIANCE Phase1/2 adult low-dose cohort completed
  Editas Medicine (Company) has completed dosing of the first cohort of adult patients with visual light perception only administered the low dose of EDIT-101. Trial enrollment remains active but has experienced a slowdown due to the ongoing impact of the COVID-19 pandemic.

- **Ocular Medicines**
  Regained full control of ocular medicines
  Editas Medicine terminated its 2017 agreement with Allergan, now part of AbbVie, and entered a new agreement with AbbVie that returned development and commercialization rights for ocular medicines to Editas Medicine. As part of the new agreement, AbbVie has transferred supplier contracts, including with the contract research organization (CRO), as well as sponsorship of the investigational new drug application (IND) for the BRILLIANCE Phase 1/2 clinical trial to Editas Medicine. The Company plans to continue to advance ocular medicines, including EDIT-101 for Leber congenital amaurosis 10 (LCA10).

***Ex Vivo* CRISPR Cell Medicines**

- **EDIT-301 for Sickle Cell Disease and Beta-Thalassemia**
  On track for IND filing by end of 2020
  Editas Medicine continues to prepare for a Phase 1/2 clinical trial evaluating EDIT-301 for the treatment of sickle cell disease. The Company has completed preclinical toxicology studies, identified a lead principal investigator, and engaged a CRO. Clinical trial materials are being manufactured by Editas Medicine. The Company remains on track to file an IND for the treatment of sickle cell disease by the end of 2020.

  Granted Rare Pediatric Disease Designation for treatment of sickle cell disease
  EDIT-301 was granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration (FDA). Under the FDA's Rare Pediatric Disease Designation and Voucher programs, a sponsor who receives approval for a drug or biologic for a rare pediatric disease may be eligible for a voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product.

  ASH data to highlight efficient editing with clinical scale manufacturing process
  Preclinical data to be presented at the 62nd American Society of Hematology Annual Meeting & Exposition (ASH) show

CRISPR/Cas12a editing efficiency of the HBG1/2 promoter exceeding 90 percent after long term engraftment in mice of healthy donor hematopoietic stem cells manufactured at clinical scale.

- **EDIT-201 to Treat Solid Tumors**
  Data demonstrating enhanced tumor killing to be presented at SITC and ASH
  EDIT-201 is comprised of healthy donor natural killer (NK) cells that have been edited using a proprietary CRISPR/Cas12a ribonucleoprotein to knock out the *CISH* and *TGFβR2* genes. At ASH, the Company will present data showing that NK cells with *CISH* and *TGFβR2* knockouts are more potent than unedited cells *in vitro*. The Company will also show that the combination of therapeutic antibodies with EDIT-201 further enhances tumor killing. Data on EDIT-201 will also be presented at the Society for Immunotherapy of Cancer's 35 th Anniversary Annual Meeting.

- **CRISPR iPSC NK (iNK) Cell Medicines to Treat Solid Tumors**
  Proprietary CRISPR/Cas12a editing of iPSCs enhances iNK tumor killing
  Preclinical data to be presented at ASH detail a CRISPR/Cas12a induced pluripotent stem cell (iPSC) editing platform. The Company will show that knockout of *CISH* and *TGFβR2* with the proprietary CRISPR/Cas12a editing platform enhances iNK cell tumor killing in a spheroid model.

Corporate

- **Intellectual Property**
  The U.S. Patent and Trademark Office (USPTO) recently issued a decision in the ongoing patent interference between the University of California, the University of Vienna, Emmanuelle Charpentier (CVC) and the Broad Institute, Inc. (Broad) regarding certain Broad CRISPR/Cas9 patents Editas Medicine exclusively licenses. The USPTO granted Broad's motion for priority benefit while denying CVC priority benefit to its two earliest provisional patent applications. As a result, Broad enters the priority phase of the interference as "Senior Party" while CVC remains the "Junior Party" for purposes of determining which entity was the first to invent the use of CRISPR/Cas9 for gene editing in eukaryotic cells. The Senior Party is presumed to be the first inventor, thus, the Junior Party, here CVC, has the burden of overcoming this presumption. The Broad patents remain valid and in force. Foundational claims covering the use of CRISPR/Cas9 for gene editing in eukaryotic cells have issued and continue to issue to Broad as patents in the United States, Europe, Japan, China, and other jurisdictions.

- **Balance Sheet**
  The Company expects that its existing cash, cash equivalents and marketable securities of $541.3 million as of September 30, 2020, and anticipated interest income will enable it to fund its operating expenses and capital expenditures into 2023.

Third Quarter 2020 Financial Results

Cash, cash equivalents, and marketable securities as of September 30, 2020, were $541.3 million, compared to $598.7 million as of June 30, 2020.

For the three months September 30, 2020, net income attributable to common stockholders was $7.8 million, or $0.12 per share, diluted, compared to net loss of $32.9 million, or $0.66 per share, for the same period in 2019.

- Collaboration and other research and development revenues increased by $59.0 million, to $62.8 million for the three months ended September 30, 2020 from $3.8 million for three months ended September 30, 2019. This increase was primarily attributable to the recognition of $59.9 million of previously deferred revenue as a result of the termination of our strategic alliance with Allergan.

- Research and development expenses increased by $11.2 million, to $33.9 million for the three months ended September 30, 2020 from $22.7 million for the three months ended September 30, 2019. The $11.2 million increase was primarily attributable to an increase in expenses related to the clinical and manufacturing development of EDIT-101 and our other programs, including a including a one-time in-process research and development expense of $5.0 million for re-acquiring the rights to EDIT-101 from Allergan. In addition, the Company continued to invest in the research and development organization which resulted in an increase in employee and facility related expenses as compared to the prior year.

- General and administrative expenses increased by $4.2 million to $19.9 million for the three months ended September 30, 2020, from $15.7 million for the same period in 2019. The $4.2 million increase was primarily attributable to an increase in expense for professional service fees incurred by the Company in connection with the termination of the Allergan agreement as well as an increase in employee and facility related expenses.

Upcoming Events

Editas Medicine plans to participate in the following scientific and medical conferences:

- Society for Immunotherapy of Cancer's 35 th Anniversary Annual Meeting, November 9-14, Virtual; and
- 62nd American Society of Hematology Annual Meeting & Exposition, December 5-8, Virtual.

Editas Medicine plans to participate in the following investor events:

- Barclays Gene Editing & Gene Therapy Summit, November 16, 10:15am EST, Virtual.

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 third quarter 2020. To access the call, please dial (844) 348-3801 (domestic) or (213) 358-0955 (international) and provide the passcode 9992536. A live webcast of the call will be available on the Investors section of the Editas Medicine website at www.editasmedicine.com and a replay will be available approximately two hours after its completion.

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 BRILLIANCE
The BRILLIANCE Phase 1/2 clinical trial of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10) is designed to assess the safety, tolerability, and efficacy of EDIT-101 in up to 18 patients with this disorder. Clinical trial sites are enrolling up to five cohorts testing up to three dose levels in this open label, multi-center study. Both adult and pediatric patients (3 – 17 years old) with a range of baseline visual acuity assessments are eligible for enrollment. Patients receive a single administration of EDIT-101 via subretinal injection in one eye. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

About EDIT-201
EDIT-201 is an experimental, allogeneic natural killer (NK) cell medicine under investigation for the treatment of solid tumor cancers. EDIT-201 is comprised of NK cells derived from pooled healthy donor blood and genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) to edit the *CISH* and *TGFβR2* genes. Editing of the *CISH* and *TGFβR2* genes is designed to overcome resistance to therapeutic antibodies and improve NK cell persistence.

About EDIT-301
EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) to edit the HBG1/2 promoter region in the beta-globin locus. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin (HbF) production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

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/Cas12a (also known as Cpf1) 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.

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 plans with respect to EDIT-101 and filing an IND for EDIT-301 by the end of 2020. 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. Those risks and uncertainties include, among other things, that the Company's expectations regarding the effects of COVID-19 may be incorrect, that data from the Company's development programs may not support registration or further development of its potential medicines due to safety, efficacy or other reasons, and other risks listed under "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 represent Company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.

EDITAS MEDICINE, INC.
Condensed Consolidated Statements of Operations
(unaudited)
(amounts in thousands, except per share and share data)

	Three Months Ended September 30,	
	2020	2019
Collaboration and other research and development revenues	$ 62,841	$ 3,848
Operating expenses:		
Research and development	33,916	22,702
General and administrative	19,936	15,734
Total operating expenses	53,852	38,436
Operating income (loss)	8,989	(34,588)
Other (expense) income, net:		
Other (expense) income, net	(1,396)	(33)
Interest income, net	226	1,680
Total other (expense) income, net	(1,170)	1,647
Net income (loss)	$ 7,819	$ (32,941)
Net income (loss) per share attributable to common stockholders, diluted	$ 0.12	$ (0.66)
Weighted-average common shares outstanding, diluted	62,697,173	49,820,455

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

	September 30, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	$ 541,291	$ 457,140
Working capital	452,011	403,881
Total assets	597,157	508,885
Deferred revenue, net of current portion	80,821	163,207
Total stockholders' equity	441,152	262,437

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