Editas Medicine Announces Third Quarter 2021 Results and Business Updates

November 8, 2021

EDIT-101 Phase 1/2 BRILLIANCE trial initial clinical data demonstrated favorable safety profile and preliminary evidence of clinical benefit; enrollment ongoing in adult high-dose and pediatric mid-dose cohorts

EDIT-301 Phase 1/2 RUBY trial for the treatment of sickle cell disease currently enrolling study participants

Presented preclinical data on novel SLEEK gene editing technology enabling high efficiency, multi-transgene knock-in in multiple clinically relevant cell types

CAMBRIDGE, Mass., , Nov. 08, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the third quarter of 2021.

"Editas recently marked an important milestone with the presentation of initial EDIT-101 results demonstrating a favorable safely profile and encouraging signals of clinical activity and evidence of ocular gene editing. Encouraged by these data, we look forward to obtaining additional clinical results, including from our ongoing adult high-dose and pediatric mid-dose cohorts," said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. "We have also continued to make excellent progress advancing our broader clinical and preclinical programs, including the expansion of our gene editing capabilities, as exemplified by our proprietary SLEEK knock-in technology, which we are already applying in our iNK program for solid tumors."

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for Leber Congenital Amaurosis 10 (LCA10), a CEP290-Related Retinal Degenerative Disorder**
  Initial clinical data demonstrated favorable safety profile, and efficacy signals in the mid-dose cohort provided initial evidence for clinical benefit
  Editas Medicine presented initial clinical data from the ongoing, open label Phase 1/2 BRILLIANCE clinical trial of EDIT-101 for the treatment of LCA10. The data included preliminary patient safety and efficacy assessments on the first two cohorts.

  No serious adverse events or dose-limiting toxicities were reported in the first six adult subjects treated with the low or mid doses of EDIT-101. In addition, early efficacy signals in the mid dose cohort provided clinical evidence of gene editing and suggest potential clinical benefits. The Company believes these initial data validate Editas Medicine's *in vivo* platform's proof of concept and provide support for the advancement of the adult high-dose cohort and the pediatric mid-dose cohort. The Company will continue to assess patients in the adult low-dose and mid-dose cohorts while it enrolls and treats patients in the adult high-dose and pediatric mid-dose cohorts. The Company remains on track to complete dosing of the adult high-dose and pediatric mid-dose cohorts in the first half of 2022.

***Ex Vivo* Gene Edited Medicines**

- **EDIT-301 for Sickle Cell Disease**
  Enrolling patients for initial dosing
  The Company is developing EDIT-301 for the treatment of sickle cell disease. Editas Medicine uses a proprietary engineered CRISPR/Cas12a ribonucleoprotein (RNP) to edit the *HBG1/2* promoter mimicking a benign and naturally occurring human fetal hemoglobin mutation. This site is a naturally validated location, as patients with Hereditary Persistence of Fetal Hemoglobin (HPFH) harbor the sickle cell mutation but do not exhibit symptoms of the disease. The Company believes that targeting this site is potentially a more effective approach with better long-term safety than editing the *BCL11A* enhancer. The Phase 1/2 RUBY trial for the treatment of sickle cell disease is currently enrolling study participants and expects to begin dosing in the first half of 2022.

- **EDIT-301 for Transfusion-Dependent Beta Thalassemia (TDT)**
  Preclinical data supporting potential treatment for TDT to be presented at ASH
  The Company will present preclinical data on EDIT-301 for the treatment of transfusion-dependent beta thalassemia at the 63rd American Society of Hematology Annual Meeting & Exposition (ASH). The data will demonstrate that edited beta thalassemia CD34+ cells show significant improvement in erythroid maturation and health, accompanied by significantly increased total hemoglobin content per cell. These data support the hypothesis that editing of the *HBG1/2* promoter using an engineered AsCas12a RNP may reverse red blood cell abnormalities associated with the disease and increase hemoglobin production. The Company believes that EDIT-301 has the potential to be an efficacious autologous cell therapy

for TDT and remains on track to file an IND by year-end.

Cellular Therapy

- **Edited iPSC NK (iNK) Cell Medicines to Treat Solid Tumors**
  Preclinical data demonstrating multiplexed gene editing and tumor clearance to be presented at SITC and ASH
  Editas Medicine is continuing to advance preclinical studies for a highly differentiated, iPSC-derived natural killer (iNK) cell medicine. At the upcoming Society for Immunotherapy of Cancer's 36 th Anniversary Annual Meeting, the Company will present data demonstrating a new method to drive high-level constitutive CD16 expression, which enhances antibody-dependent cell-mediated cytotoxicity (ADCC) of iNK cells, enabling improved serial tumor killing. This was achieved through transgene knock-in using the Company's engineered AsCas12a and new SLEEK (SeLection by Essential-gene Exon Knock-in) gene editing technology. These data provide evidence for the potential of the iNK program to exert enhanced anti-tumor activity in the clinic.

  At ASH, the Company will present data showing that iNK cells, edited with CRISPR/Cas12a to knock out CISH and TGFβR2, demonstrated robust anti-tumor efficacy resulting in a three-fold reduction in tumor burden compared to unedited iNK cell treatment. This potent anti-tumor activity is maintained after cryopreservation and supports further development, potentially with additional gene edits, of the iNK program as a potent allogeneic cell-based medicine for cancer.

- **Alpha-Beta T Cells for Oncology**
  Bristol Myers Squibb opting into additional program
  Editas Medicine and Bristol Myers Squibb (BMS) continue to advance alpha-beta T cell medicines for the treatment of solid and liquid tumors. BMS recently opted into an additional gene editing program, further validating the Company's technology and expertise in engineered cell medicines. This marks the fourth program, one of which has advanced to development candidate status, opted into by BMS in the last twelve months.

Gene Editing Platform

Editas Medicine presented preclinical data at the Cold Spring Harbor Laboratory's Genome Engineering: CRISPR Frontiers meeting on its SLEEK gene editing technology. The data demonstrated that SLEEK, which utilizes the Editas-engineered AsCas12a nuclease, resulted in the knock-in of multiple clinically relevant transgenes through a proprietary process that selects for cells containing the knock-in cargo. More than 90 percent knock-in efficiencies were observed in various clinically relevant target cells, including iPSCs, T cells, and NK cells. Additionally, SLEEK may be used to fine-tune the expression levels of transgene cargos, an important attribute of next-generation cell therapy medicines.

The Company also presented preclinical data at the TIDES Oligonucleotide and Peptide Therapeutics Conference demonstrating the editing and manufacturing advantages of the AsCas12a nuclease. Specifically, the data showed that AsCas12a achieves high editing rates across multiple cell types, highly efficient multiplexed editing, single and dual site-specific knock-ins, and has higher intrinsic specificity and sequence fidelity due to its shorter chemically synthesized guide RNA.

Corporate

- **Leadership**
  In September, Editas Medicine appointed Emma Reeve to its Board of Directors and as Chairperson of the Audit Committee. Ms. Reeve is an accomplished biopharmaceutical executive with more than 25 years of global financial experience across pharmaceutical, medical device, and bio-pharma companies. Most recently, Ms. Reeve served as Chief Financial Officer of Constellation Pharmaceuticals, Inc., a development-stage oncology company, prior to its acquisition by MorphoSys AG in 2021. Prior to Constellation, Ms. Reeve acted as interim Chief Financial Officer and Corporate Controller of Parexel International, a global biopharmaceutical services company. Earlier in her career, Ms. Reeve served as Chief Financial Officer of both Inotek Pharmaceuticals and Aton Pharma. Additionally, she held senior finance and operational roles at Bristol-Myers Squibb, Merck, and Novartis.

  In October, the Company also appointed Bernadette Connaughton to its Board of Directors. Ms. Connaughton is an accomplished pharmaceutical executive with more than 30 years of global strategic, commercial, and biopharmaceutical industry expertise. Ms. Connaughton spent her career at Bristol-Myers Squibb, building a consistent track record of achieving sales growth, improving operational models, and increasing profitability in the U.S., Europe, and Asia. She most recently served as President Intercontinental. In this role, she developed successful, multi-year commercialization strategies for several oncology, virology, and immunology products.

Third Quarter 2021 Financial Results

- Cash, cash equivalents, and marketable securities as of September 30, 2021, were $657.0 million, compared to $698.1 million as of June 30, 2021. The Company expects that its existing cash, cash equivalents and marketable securities will enable it to fund its operating expenses and capital expenditures well into 2023.

- For the three months ended September 30, 2021, net loss attributable to common stockholders was $39.1 million, or $0.57 per share, compared to net income of $7.8 million, or $0.13 per share, for the same period in 2020.

- Collaboration and other research and development revenues were $6.2 million for the three months ended September 30, 2021, compared to $62.8 million for the same period in 2020. The $56.6 million decrease 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 during the third quarter 2020 for which there was no similar revenue recognized in the third quarter of 2021.

- Research and development expenses decreased by $4.7 million to $29.2 million for the three months ended September 30, 2021, from $33.9 million for the same period in 2020. The $4.7 million decrease related primarily to a one-time in-process research and development expense of $5.0 million for re-acquiring the rights to EDIT-101 from Allergan in the third quarter of 2020 for which there was no similar expense in the third quarter of 2021.

- General and administrative expenses decreased by $3.7 million to $16.2 million for the three months ended September 30, 2021, from $19.9 million for the same period in 2020. The $3.7 million decrease was primarily attributable to decreased professional service expenses during the third quarter of 2021.

Upcoming Events

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

- Society for Immunotherapy of Cancer (SITC) 36th Annual Meeting, November 10 - 14, Washington, D.C.
- 63rd American Society of Hematology (ASH) Annual Meeting & Exposition, December 11 - 14, Atlanta, Georgia

Editas Medicine plans to participate in the following investor events:

- Barclays Gene Editing & Gene Therapy Summit, November 15, Virtual
- 4th Annual Evercore ISI HealthCONx Conference, December 2, Virtual

Conference Call
The Editas Medicine management team will host a conference call and webcast today at 8:00 a.m. ET to provide and discuss a corporate update and financial results for the second quarter 2021. To access the call, please dial 877-407-0989 (domestic) or 201-389-0921 (international) and ask for the Editas Medicine earnings call. A live webcast of the call will also 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 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.

About EDIT-101
EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10), a CEP290-related retinal degenerative disorder. 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. Patients are monitored every three months for a year after dosing and less frequently for an additional two years thereafter. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

About EDIT-301
EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT). EDIT-301 is comprised of sickle patient (for SCD) or normal donor (for TDT) CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) that targets the HBG1 and HBG2 promoters in the beta-globin locus where naturally occurring fetal hemoglobin (HbF) inducing mutations reside. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in HbF production, which has the potential to provide a one-time, durable treatment benefit for people living with sickle cell disease and TDT.

About RUBY
The RUBY Trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in patients with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301. Additional details are available on www.clinicaltrials.gov (NCT#04853576).

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 initiation, timing, progress and results of the Company’s preclinical and clinical studies and its research and development programs, including completing dosing of both the adult high-dose and pediatric mid-dose cohorts in the first half of 2022 and beginning patient dosing in the RUBY trial in the first half of 2022, the timing for the Company’s receipt and presentation of data from its clinical trials and preclinical studies, the timing or likelihood of regulatory filings and approvals, including filing an IND for EDIT-301 for the treatment of transfusion-dependent beta thalassemia by the end of 2021, and the Company’s expectations regarding cash runway. 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 pre-clinical studies and clinical trials, including the BRILLIANCE and RUBY trials, and clinical development of the Company’s product candidates; availability and timing of results from pre-clinical 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, as updated by the Company’s subsequent filings 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,	
	2021	**2020**
Collaboration and other research and development revenues	$ 6,197	$ 62,841
Operating expenses:		
Research and development	29,265	33,916
General and administrative	16,185	19,936
Total operating expenses	45,450	53,852
Operating (loss) income:	(39,253)	8,989
Other income (expense), net:		
Other income (expense), net	19	(1,396)
Interest income, net	152	226
Total other income (expense), net	171	(1,170)
Net (loss) income	$ (39,082)	$ 7,819
Net (loss) income per share, basic	$ (0.57)	$ 0.13
Net (loss) income per share, diluted	$ (0.57)	$ 0.12
Weighted-average common shares outstanding, basic	68,219,742	62,144,118
Weighted-average common shares outstanding, diluted	68,219,742	62,697,173

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

	September 30, 2021	**December 31, 2020**
Cash, cash equivalents, and marketable securities	$ 657,039	$ 511,774
Working capital	516,701	360,879
Total assets	717,019	572,602
Deferred revenue, net of current portion	60,888	73,984
Total stockholders’ equity	586,794	393,586

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Source: Editas Medicine, Inc.