Editas Medicine Announces Second Quarter 2021 Results and Business Updates

August 4, 2021

Enrolling first pediatric and adult high dose cohorts of EDIT-101 BRILLIANCE trial for LCA10

EDIT-101 initial clinical data planned for September 2021

EDIT-301 RUBY trial for sickle cell disease screening patients; first patient on track to be dosed by year-end

Strengthened leadership with appointment of Chi Li as Chief Regulatory Officer and Bruce Eaton as Chief Business Officer

CAMBRIDGE, Mass., Aug. 04, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the second quarter of 2021.

"We continue to make exciting progress on our clinical and preclinical programs, as well as significant advancements in our underlying gene editing platform," said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. "The BRILLIANCE trial for EDIT-101 is now enrolling the first pediatric and adult high-dose cohorts, and we look forward to presenting our Company's first clinical data next month. Patient screening is underway for our RUBY trial of EDIT-301 for sickle cell disease, and we expect to dose our first patient by year end. In addition, we enhanced the technological capabilities of our novel engineered nucleases, and further advanced our earlier stage pipeline, including the iPSC-derived NK platform."

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for Leber Congenital Amaurosis 10 (LCA10)**
  IDMC endorsed proceeding with first pediatric cohort based on a review of clinical safety data; concurrently enrolling patients in adult high-dose cohort
  Editas Medicine has completed dosing of the adult mid-dose cohort and, following the endorsement by the Independent Data Monitoring Committee (IDMC), has opened enrollment for the first pediatric cohort. The Company is also enrolling patients in the adult high-dose cohort. Completion of dosing for both cohorts is expected in the first half of 2022.

  Initial clinical data planned for the International Symposium on Retinal Degeneration (RD2021) in September 2021
  Initial EDIT-101 clinical data is planned for presentation at the International Symposium on Retinal Degeneration (RD2021) in September 2021 by one of the study's Principal Investigators. Data will include patient safety assessments and a preliminary analysis of secondary endpoints relating to signals of gene editing and clinical benefit. The presentation will cover cumulative data from patients in the adult low-dose and mid-dose cohorts. As required by the trial protocol, all patients are monitored every three months for the first year and at various timepoints for another two years.

***Ex Vivo* Gene Edited Medicines**

- **EDIT-301 for Sickle Cell Disease**
  On track to dose first patient in RUBY trial by year-end
  The Company is developing EDIT-301 as a potentially best-in-class medicine to treat 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 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 screening study participants. The Company remains on track to begin patient dosing in the RUBY trial by the end of 2021.

  Health Canada approved CTA for trial initiation in Canada
  The Company received an approved clinical trial application (CTA) from Health Canada for the RUBY trial of EDIT-301, expanding the number of potential clinical sites for the study. This is Editas Medicine's first CTA approval.

  Preclinical data presented at EHA supports EDIT-301 as potential best-in-class treatment for sickle cell disease
  Editas Medicine presented preclinical data on EDIT-301 at the European Hematology Association Congress (EHA). The data demonstrated therapeutically relevant levels of fetal hemoglobin (HbF) in red blood cells derived from sickle cell patient CD34+ cells, as well as a reduction of sickling and improved rheological behavior. Cells were edited with a novel, highly active and specific, Editas-engineered AsCas12a enzyme with no detection of off-target editing. These data further

support EDIT-301 for clinical development use.

- **EDIT-301 for Beta-Thalassemia**
  On track to file IND by year-end for a differentiated treatment for beta-thalassemia
  The Company remains on track to file an investigational new drug application for EDIT-301 for the treatment of beta-thalassemia by the end of 2021.

Cellular Therapy

- **Edited iPSC NK (iNK) Cell Medicines to Treat Solid Tumors**
  Advancing preclinical studies towards multiplexed edited product
  Editas Medicine is continuing to advance preclinical studies for a highly differentiated, iPSC-derived natural killer (NK) cell medicine. The Company is optimizing editing configurations for corresponding clinical targets. The finalized product candidate will include multiple edits, for which the Company has previously demonstrated high levels of multiplexed editing efficiency.

Corporate

- **Leadership**
  Editas Medicine announced the appointment of Chi Li, Ph.D., MBA, RAC, as the Company's Senior Vice President and Chief Regulatory Officer. Dr. Li brings to Editas Medicine more than 20 years of experience in drug development and global/US regulatory affairs at major biotechnology and pharmaceutical companies. He played an important and strategic role in the development, submission, and approval of several new drugs and biologics across a number of therapeutic areas, globally. Dr. Li joins Editas Medicine from Celularity where he served as Chief Regulatory Officer. Previously, Dr. Li served as Vice President, Regulatory Affairs at both Allergan and Bayer. Dr. Li earned a Ph.D. in organic chemistry from Purdue University and an MBA from Rutgers University. He holds a Regulatory Affairs Certification from the Regulatory Affairs Professionals Society.

  The Company announced the promotion of Bruce E. Eaton, Ph.D., to Executive Vice President and Chief Business Officer. Dr. Eaton has worked alongside Editas Medicine since 2015, first as a consultant, then as a research collaborator, and officially joining as Senior Vice President, Chemistry, and Site Head, Boulder, in January 2018 following Editas Medicine's acquisition of assets from i2 Pharmaceuticals, where he was Founder and CEO. Dr. Eaton has more than 30 years of biotech scientific, operations, business development, and corporate strategy experience in both public and private companies. He has a deep understanding of biochemistry and biophysics for the innovation and development of medicines. Throughout his career, Dr. Eaton has founded multiple companies in both life sciences and human therapeutics and has served in C-level and director level roles. In addition, he has acted as an advisor to numerous government and academic research institutions.

- **Gene Editing Platform**
  In collaboration with Integrated DNA Technologies, Inc. (IDT), a leading comprehensive genomics research solutions provider, Editas Medicine published data on an engineered "AsCas12a Ultra" nuclease in *Nature Communications* that demonstrated substantial improvement in editing efficiency compared to wild type AsCas12a while retaining its high specificity. Editas believes its engineered AsCas12a, which leverages the beneficial features of AsCas12a Ultra, is a best-in-class nuclease, increasing access to the genome and reducing off-target editing risk associated with other CRISPR nucleases. This Editas-engineered AsCas12a nuclease is being utilized in the EDIT-301 experimental medicine for the treatment of sickle cell disease, iNK programs for solid tumors, and other undisclosed programs.

  The Company plans to present data on a new methodology it refers to as SLEEK (SeLection by Essential-gene Exon Knock-in) technology at the upcoming Cold Spring Harbor Genome Engineering: CRISPR Frontiers meeting. This technology enables robust knock-in efficiencies using induced Pluripotent Stem Cells (iPSCs), T cells, and Natural Killer (NK) cells. This method simplifies iPSC clone selection process by increasing rates of knock-in and subsequent single-cell cloning. Editas Medicine believes SLEEK is the optimal strategy for achieving robust multi-transgene knock-in for the next generation cell-based medicines.

- **Manufacturing**
  Editas Medicine continues to advance internal and external manufacturing capabilities for the Company's portfolio of *in vivo* gene edited medicines, *ex vivo* gene edited cell medicines, and cell therapy medicines. The Company has achieved clinical manufacturing readiness for the RUBY clinical trial for EDIT-301 with assays ready for distribution. The Company has also defined a Chemistry, Manufacturing and Controls supply strategy for North America, further streamlining the Company's manufacturing capabilities.

Second Quarter 2021 Financial Results

- Cash, cash equivalents, and marketable securities as of June 30, 2021, were $698.1 million, compared to $723.2 million as of March 31, 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 June 30, 2021, net loss attributable to common stockholders was $55.3 million, or $0.81 per share, compared to $23.6 million, or $0.43 per share, for the same period in 2020.

- Collaboration and other research and development revenues were $0.4 million for the three months ended June 30, 2021, compared to $10.7 million for the same period in 2020. The $10.3 million decrease was primarily attributable to $7.6 million in revenue recognized pursuant to an out-license agreement we entered into during the second quarter of 2020 and a $0.8 million increase in revenue recognized related to our strategic alliance with Allergan for which there was no similar revenue recognized in the second quarter of 2021.

- Research and development expenses increased by $5.8 million to $33.8 million for the three months ended June 30, 2021, from $28.0 million for the same period in 2020. The $5.8 million increase was primarily attributable to increased manufacturing and clinical related costs during the second quarter of 2021.

- General and administrative expenses increased by $7.9 million to $22.0 million for the three months ended June 30, 2021, from $14.1 million for the same period in 2020. The $7.9 million increase was primarily attributable to increased stock-based compensation expense during the second quarter of 2021.

Upcoming Events

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

- Cold Spring Harbor Laboratory (CSHL) – Genome Engineering: CRISPR Frontiers, August 18 - 20, Virtual
- World Orphan Drug Congress USA, August 25-27, Oxon Hill, MD
- 19th Annual International Symposium on Retinal Degeneration (RD2021), September 28 - October 2, Nashville, TN

Editas Medicine plans to participate in the following investor events:

- 2021 Wells Fargo Healthcare Conference, September 10, Virtual
- Morgan Stanley 18th Annual Global Healthcare Conference, September 14, Virtual
- Chardan's 5th Annual Genetic Medicines Conference, October 4, 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). 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 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). 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-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 proprietary highly specific and efficient engineered CRISPR/Cas12a 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 durable treatment benefit for people living with sickle cell disease.

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 pediatric and the adult high-dose cohorts in the BRILLIANCE trial in the first half of 2022 and beginning patient dosing in the RUBY trial by the end of 2021, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including presenting initial clinical data from the BRILLIANCE trial at RD2021 in September 2021, and the timing or likelihood of regulatory filings and approvals, including filing an IND for EDIT-301 for the treatment of beta-thalassemia by the end of 2021. 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 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 June 30,	
	2021	**2020**
Collaboration and other research and development revenues	$ 379	$ 10,749
Operating expenses:		
Research and development	33,753	28,007
General and administrative	22,027	14,081
Total operating expenses	55,780	42,088
Operating loss	(55,401)	(31,339)
Other income, net:		
Other (expense) income, net	(1)	7,175
Interest income, net	146	592
Total other income, net	145	7,767
Net loss	$ (55,256)	$ (23,572)
Net loss per share basic and diluted	$ (0.81)	$ (0.43)
Weighted-average common shares outstanding, basic and diluted	67,877,126	55,346,052

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

	June 30, 2021	**December 31, 2020**
Cash, cash equivalents, and marketable securities	$ 698,137	$ 511,774
Working capital	550,276	360,879
Total assets	755,880	572,602
Deferred revenue, net of current portion	55,221	73,984
Total stockholders' equity	613,361	393,586

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

Investors
Ron Moldaver
(617) 401-9052
ir@editasmed.com

Source: Editas Medicine, Inc.