Editas Medicine Announces First Quarter 2022 Results and Business Updates

May 4, 2022

Appointed Gilmore O'Neill as CEO effective June 1, 2022; James C. Mullen to serve as Executive Chairman

First pediatric patient dosed in Phase 1/2 BRILLIANCE trial of EDIT-101 for LCA10; clinical data update expected in 2H 2022

On track to dose first SCD patient in 1H 2022 and first TDT patient by year-end with EDIT-301; initial SCD data expected by year-end

Preclinical data demonstrated that edited iNK cells increased persistence, enhanced anti-tumor activity, and extended survival, supporting continued development as a novel therapeutic approach for solid tumors

Favorable USPTO patent interference decision issued to The Broad Institute affirming foundational CRISPR/Cas9 intellectual property

CAMBRIDGE, Mass., May 04, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the first quarter of 2022.

"I am delighted with the appointment of Gilmore O'Neill as our incoming CEO," said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. "Gilmore brings significant drug development experience, proven leadership, and passion for genetic medicine to Editas, and I look forward to closely collaborating with him and the rest of the management team as Executive Chairman."

Mr. Mullen continued, "We're excited by the advances in our pipeline programs, including the important milestone of dosing our first pediatric patient in the BRILLIANCE study of EDIT-101 for LCA10. This represents the first-ever dosing of a pediatric patient with an *in vivo* CRISPR gene editing medicine. We look forward to important clinical and preclinical updates this year across our ocular, hematology, and oncology programs. In addition, the recent USPTO decision reaffirms the strength of our foundational intellectual property as the CRISPR landscape matures."

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for LCA10**
  First pediatric patient dosed in BRILLIANCE study; on track for clinical data update in the second half of 2022
  Editas Medicine is developing EDIT-101 for the treatment for Leber Congenital Amaurosis 10 (LCA10), a CEP290-related retinal degenerative disorder. Previously announced preliminary EDIT-101 clinical results demonstrated a favorable safety profile and encouraging signals of clinical benefit.
  - In April 2022, the Company announced treatment of the first pediatric patient in the BRILLIANCE trial, marking the world's first *in vivo* dosing of a pediatric patient with a CRISPR gene editing medicine. The Company expects to complete dosing of the pediatric mid-dose cohort in its BRILLIANCE study of EDIT-101 in the first half of 2022, and to initiate dosing of the pediatric high-dose cohort later this year.
  - At the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Denver, Editas Medicine presented data demonstrating that EDIT-101 viral shedding was transient, with no indication of systemic viral persistence following EDIT-101 administration. No correlation between dose and levels of EDIT-101 viral shedding was observed, further supporting a favorable safety profile for EDIT-101.
  - Editas Medicine expects to provide a clinical update on the BRILLIANCE trial in the second half of 2022 including safety and efficacy assessments on all patients who have had at least six months of follow-up evaluations, which will include at least 12 months of data on the adult mid-dose cohort, and at least six months of data on the adult high-dose cohort.
  - Additionally, the Company is expanding enrollment in one or more of the previously completed adult cohorts to explore dose response and support establishment of registrational trial endpoints, which are anticipated by year-end.

- **EDIT-103 for RHO-adRP**
  Preclinical data to be presented today at ARVO; program advancing towards IND-enabling studies
  Editas Medicine is developing EDIT-103 for the treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP), a progressive form of retinal degeneration. EDIT-103 uses two adeno-associated virus (AAV) vectors to knock out and replace mutations in the rhodopsin gene in order to preserve photoreceptor function. This approach can potentially address more than 150 gene mutations that cause RHO-adRP. The Company is continuing to advance EDIT-103 towards IND-enabling studies.
  - Later today, Editas Medicine will present preclinical data for EDIT-103 during an oral presentation at the ARVO Annual Meeting in Denver. The presentation can be accessed on the Company's Posters & Presentations section

of the website at 1:08 p.m. ET today.

***Ex Vivo* Gene Edited Medicines**

- **EDIT-301 for Sickle Cell Disease**
  On track for first patient dosing in 1H 2022 and topline data by year-end
  The Company is developing EDIT-301 for the treatment of sickle cell disease (SCD). Following human genetics, Editas is editing the HBG1/2 promoter and disrupting the binding site of BCL11a, consistent with observed naturally occurring protective mutations. The Company believes that targeting the HBG1/2 promoter site using a proprietary Editas-engineered AsCas12a enzyme is potentially a more effective approach with better long-term safety than other editing targets and mechanisms.
    - The Phase 1/2 RUBY trial for the treatment of SCD is currently enrolling study participants and is on track to begin dosing in the first half of 2022, with initial clinical results expected by year-end.

- **EDIT-301 for TDT**
  Received FDA Rare Pediatric Disease Designation; first patient dosed expected in 2022
  Editas Medicine is also developing EDIT-301 for the treatment of transfusion-dependent beta thalassemia (TDT).
    - The U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease designation to EDIT-301, an investigational, gene-edited medicine for the treatment of TDT. The FDA previously granted Rare Pediatric Disease designation to EDIT-301 for the treatment of SCD.
    - Preparations to initiate the Phase 1/2 clinical trial designed to assess the safety, tolerability, and preliminary efficacy of EDIT-301 for the treatment of TDT are underway. The Company expects to dose the first TDT patient in 2022.

Cellular Therapy

- **EDIT-202 Multiplexed iNK for Solid Tumors**
  Preclinical data demonstrate enhanced anti-tumor activity, extended survival and increased persistence
  Editas Medicine is developing EDIT-202, a highly differentiated, iPSC-derived natural killer cell (iNK) investigational medicine with double knock-in and double knock-out edits. This approach has the potential to create an allogeneic "off-the-shelf" NK cell therapy medicine with enhanced activity against solid tumors.
    - At the American Association for Cancer Research conference in April, Editas Medicine reported *in vitro* and *in vivo* preclinical data on the enhanced tumor killing capacity of modified iNK cell therapies using Editas Medicine's proprietary AsCas12a gene editing enzyme. Edited iNK cells combined with trastuzumab significantly reduced tumor burden and increased survival over wild type iNK cells plus trastuzumab, and edited iNKs survived for a prolonged period without exogenous cytokine support.
    - At the upcoming American Society for Gene and Cell Therapy (ASGCT) Annual Meeting, the Company will present *in vitro* and *in vivo* preclinical data on EDIT-202 demonstrating significantly augmented antibody-dependent cellular cytotoxicity (ADCC) against multiple solid tumor cell lines and prolonged *in vitro* persistence in the absence of exogenous cytokines. EDIT-202 combined with trastuzumab resulted in greater reduction in tumor burden and prolonged survival compared with trastuzumab alone or trastuzumab plus wild-type iNK cells in an ovarian cancer mouse model.

- **Alpha-Beta T Cells for Oncology**
  Bristol Myers Squibb opts into seventh alpha-beta T cell program
  The ongoing collaboration between Editas Medicine and Bristol Myers Squibb (BMS) continues to advance alpha-beta T cell investigational medicines for the treatment of solid and liquid tumors, leveraging Editas Medicine's unique platform technologies, including Cas9 and AsCas12a. BMS recently opted into an additional gene editing program, further validating the Company's technology and expertise in cellular therapies. This marks the seventh program opted into by BMS since the start of the collaboration, one of which has advanced to development candidate status.

Corporate

- **Leadership**
  Gilmore O'Neill to serve as Chief Executive Officer and James Mullen as Executive Chairman starting June 1, 2022
  Editas Medicine has appointed Dr. Gilmore O'Neill, M.B., M.M.Sc., as President and Chief Executive Officer. James C. Mullen will serve as Executive Chairman of the Company's Board of Directors. These changes will be effective on June 1, 2022. Dr. O'Neill brings to Editas Medicine nearly 20 years of experience in genetic medicine, neurobiology, and clinical development. Dr. O'Neill also has a track record of driving and leading several of biotech's most successful clinical programs and achieving marketing approvals for several medicines. He most recently served as Executive Vice President of R&D and Chief Medical Officer at Sarepta Therapeutics. During his tenure at Sarepta, he led the R&D leadership team that was accountable for creating and driving discovery, preclinical and clinical development, and global regulatory strategy of its RNA and gene therapeutic portfolio. Prior to that, Dr. O'Neill held several leadership roles at Biogen over a 15-year

period, most recently serving as Senior Vice President responsible for all late-stage clinical development. Dr. O'Neill received a Bachelor of Medicine degree from University College Dublin and a Master of Medical Sciences degree from Harvard University. He is licensed to practice medicine in the Commonwealth of Massachusetts.

- **Intellectual Property**
  Favorable decision from USPTO in CRISPR patent interference
  In February 2022, the U.S. Patent and Trademark Office (USPTO) issued a favorable decision to The Broad Institute, Inc. (Broad) involving specific patents for CRISPR/Cas9 editing in human cells. The patents at issue are owned by Broad and exclusively licensed to Editas Medicine for the development of medicines for people living with serious diseases. The losing party, the University of California, University of Vienna and Emmanuelle Charpentier (CVC), has filed an appeal to the Court of Appeals for the Federal Circuit. The Company anticipates that Broad will prevail on appeal. The action by the USPTO is the second favorable decision determining Broad as the first group to invent the use of CRISPR/Cas9 for editing DNA in those cells necessary for making gene editing medicines for people.
    - This decision by the USPTO reinforces the Company's strong intellectual property position. Editas Medicine's in-licensed patents broadly cover CRISPR/Cas9 and CRISPR/Cas12a gene editing in all human cells. Successfully editing this cell type is essential to making CRISPR-based medicines.
    - As the exclusive licensee of both Cas9 and Cas12a patent estates from Broad, the Company is able to provide exclusive and non-exclusive sublicenses for Cas9, Cas12a, and other patents held by the Editas Medicine.

- **Manufacturing**
  EDIT-202 development process ensures pure final population of iPSC clones
  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 continued development of a scalable, feeder-free process of GMP iNK cell production. For the EDIT-202 program, the four gene edits were successfully made in iPSCs derived from a clinical master cell bank with high efficiency using the Company's proprietary AsCas12a enzyme. Editas Medicine believes this to be a critical safety process since these clones have been fully characterized by sequencing and cytogenetic analyses, allowing selected clones in the primary cell bank to contain only the desired edits, thereby ensuring a pure final population of iPSCs.

First Quarter 2022 Financial Results

- Cash, cash equivalents, and marketable securities as of March 31, 2022, were $566.4 million, compared to $619.9 million as of December 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 into early 2024.

- For the three months ended March 31, 2022, net loss attributable to common stockholders was $50.5 million, or $0.74 per share, compared to net loss of $56.7 million, or $0.86 per share, for the same period in 2021.

- Collaboration and other research and development revenues were $6.8 million for the three months ended March 31, 2022, compared to $6.5 million for the same period in 2021. The increase was primarily attributable to additional programs licensed under the Company's collaboration with BMS in 2022.

- Research and development expenses decreased by $3.9 million to $38.0 million for the three months ended March 31, 2022, from $41.9 million for the same period in 2021. The decrease primarily related to success payments that were achieved under certain of the Company's license agreements during the first quarter of 2021 for which there was no similar activity during the first quarter of 2022. This decrease was partially offset by increased employee-related expenses and increased manufacturing and clinical-related expenses related to the Company's ongoing clinical trials.

- General and administrative expenses decreased by $1.9 million to $19.5 million for the three months ended March 31, 2022, from $21.4 million for the same period in 2021. The decrease was primarily attributable to the Company incurring lower intellectual property legal fees related to the prosecution and maintenance of the Company's patents.

Upcoming Events

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

- TIDES: Oligonucleotide & Peptide Therapeutics Annual Meeting
  May 9-12, 2022, Boston, MA

- American Society for Gene and Cell Therapy Annual Meeting
  May 16-19, 2022, Washington, DC

Editas Medicine plans to participate in the following investor events:

- RBC Capital Markets Global Healthcare Conference 2022
  May 17, 2022, New York, NY

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 first quarter of 2022. 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 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/Cas9-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. EDIT-101 has been granted Rare Pediatric Disease and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) and Orphan Designation from the European Medicines Agency (EMA).

About BRILLIANCE
The BRILLIANCE Phase 1/2 clinical trial of EDIT-101 for the treatment of LCA10 is designed to assess the safety, tolerability, and efficacy of EDIT-101 in 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 cell therapy medicine under investigation for the treatment of severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT). EDIT-301 consists of patient-derived CD34+ hematopoietic stem and progenitor cells edited at the gamma globin gene (HBG1 and HBG2) promoters, where naturally occurring fetal hemoglobin (HbF) inducing mutations reside, by a highly specific and efficient proprietary engineered AsCas12a nuclease. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin production, which has the potential to provide a one-time, durable treatment benefit for people living with severe SCD 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 the pediatric mid-dose cohort in the BRILLIANCE trial in the first half of 2022, initiating dosing of the pediatric high-dose cohort in 2022, and establishing registrational trial criteria by year-end 2022, dosing the first patient in the RUBY trial in the first half of 2022, and dosing the first TDT patient with EDIT-301 in 2022, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including a clinical update on the BRILLIANCE trial in the second half of 2022 and the initial clinical data from the RUBY trial by year-end 2022, the timing or likelihood of regulatory filings and approvals, the Company's expectations regarding cash runway, and the Company's expectations regarding the appeal of the USPTO decision on the patents it in-licenses from Broad. 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; availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; and uncertainties and unpredictable outcomes inherent in adversarial proceedings and litigation regarding intellectual property rights, including the appeal of the USPTO decision in favor of Broad and the impact on the Company of any loss by Broad in such appeal. 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.
Consolidated Statement of Operations
(amounts in thousands, except share and per share data)
(Unaudited)

	Three Months Ended March 31,

	2022	2021
Collaboration and other research and development revenues	$ 6,771	$ 6,499
Operating expenses:		
Research and development	37,976	41,937
General and administrative	19,545	21,445
Total operating expenses	57,521	63,382
Operating loss	(50,750)	(56,883)
Other income, net:		
Other (expense) income, net	(234)	21
Interest income, net	469	134
Total other income, net	235	155
Net loss	$ (50,515)	$ (56,728)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.74)	$ (0.86)
Weighted-average common shares outstanding, basic and diluted	68,484,978	65,992,395

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

	March 31, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 566,408	$ 619,916
Working capital	460,528	460,426
Total assets	623,108	677,483
Deferred revenue, net of current portion	60,888	60,888
Total stockholders' equity	512,760	553,642

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