Editas Medicine Announces Fourth Quarter and Full Year 2021 Results and Business Updates

February 24, 2022

EDIT-101 Phase 1/2 BRILLIANCE trial enrolling mid-dose pediatric cohort; clinical data update expected in 2H 2022

EDIT-301 remains on track to dose first sickle cell disease patient in 1H 2022 and first TDT patient in 2022

EDIT-103 for RHO-adRP and EDIT-202 for solid tumors advancing towards IND-enabling studies

CAMBRIDGE, Mass., Feb. 24, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the fourth quarter and full year 2021.

"We're entering 2022 with an expanded pipeline of clinical programs and new preclinical candidates, enabled by our best-in-class gene editing technologies," said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. "In 2021, we demonstrated the ability to edit *in vivo* with EDIT-101, supporting continued development and additional ocular opportunities such as EDIT-103 for retinitis pigmentosa, as well as opportunities in other organs. In 2022, we expect to release additional data for EDIT-101, show initial clinical findings for EDIT-301 in sickle cell disease, and begin patient dosing in the beta thalassemia trial. We also expect to begin IND-enabling studies for EDIT-202, the first for our iNK programs."

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for LCA10**
  First ever in vivo *ocular gene editing data demonstrated evidence of gene editing and potential clinical benefit; clinical update expected in 2H 2022*
  Editas Medicine completed dosing of all adult cohorts in its BRILLIANCE study of EDIT-101, an investigational 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.
  - The Company remains on track to complete dosing of the pediatric mid-dose cohort in the first half of 2022 and expects to initiate dosing of the pediatric high-dose cohort this year.
  - 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.
  - Editas Medicine also expects to provide a clinical update on the BRILLIANCE trial in the second half of 2022. The update is expected to provide 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.
  - At the upcoming Association for Research in Vision and Ophthalmology (ARVO) annual meeting, the Company will provide a safety profile update on EDIT-101, evaluating viral shedding in the BRILLIANCE trial to determine any risk of systematic viral persistence.

- **EDIT-103 for RHO-adRP**
  Advancing towards IND enabling studies; additional non-human primate data to be presented at ARVO
  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 believes that simultaneously knocking out and replacing a gene in the same cell *in vivo* may lead to addressing other autosomal dominant diseases, where gain of negative function requires correction.
  - Editas Medicine has shown that non-human primate data for EDIT-103 demonstrated more than 95% productive editing and potentially therapeutically effective restoration of normal rhodopsin protein. The program continues to advance towards IND-enabling studies, with additional preclinical data to be presented at the upcoming ARVO annual meeting.

- **EDIT-102 for USH2A**
  Productive editing enhanced by approximately 350% compared to initial construct
  An optimized version of EDIT-102 for treatment of Usher syndrome 2A (USH2A)-associated retinitis pigmentosa is in lead optimization. Using a dual-vector approach with an AsCas12a nuclease, Editas Medicine has enhanced productive editing

by approximately 350% compared to the initial construct.

- Editas Medicine expects to declare an additional new development candidate for an *in vivo* ocular indication in 2022.

Ex Vivo Gene Edited Medicines

- **EDIT-301 for Sickle Cell Disease**
  Naturally de-risked approach supported by highly specific and proprietary AsCas12a enzyme; first patient dosing expected in 1H 2022 and initial data expected by year-end
  The Company is developing EDIT-301 for the treatment of sickle cell disease. Following human genetics, Editas is editing the HBG1/2 promoter and disrupting the binding site of BCL11a, consistent with observed naturally occurring human mutations. These mutations mimic the asymptomatic condition Hereditary Persistence of Fetal Hemoglobin (HPFH), thereby ameliorating disease symptoms. EDIT-301 was specifically designed using Editas Medicine's proprietary engineered AsCas12a enzyme to generate high efficiency edits in multipotent long-term hematopoietic stem cells for sustained efficacy and durability. The Company believes that targeting the HBG1/2 promoter site 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 sickle cell disease 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**
  IND approved; first patient dosed expected in 2022
  Editas Medicine is also developing EDIT-301 for the treatment of transfusion-dependent beta thalassemia (TDT), for which the IND was cleared by the U.S. Food and Drug Administration (FDA) in December 2021. Preparations to initiate a 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**
  New preclinical data to be presented at AACR
  Editas Medicine has announced the development candidate EDIT-202, a highly differentiated, iPSC-derived natural killer cell (iNK) investigational medicine with double knock-in and double knock-out edits. These edits serve to enhance adaptive immune response and improve cell proliferation, cytolytic activity and persistence, as well as overcome suppressive tumor microenvironments. This approach has the potential to create an allogeneic "off-the-shelf" NK cell therapy medicine with enhanced activity against solid tumors.
    - Editas Medicine expects to advance this therapy in preclinical development during 2022. Additional *in vitro* and *in vivo* preclinical data supporting the development of EDIT-202 as a potential allogeneic cell-based medicine for treating solid tumors will be presented at the next American Associate for Cancer Research (AACR) annual meeting.

- **Alpha-Beta T Cells for Oncology**
  Six ongoing programs including one development candidate
  The ongoing collaboration between Editas Medicine and Bristol Myers Squibb (BMS) continues to advance alpha-beta T cell medicines for the treatment of solid and liquid tumors, leveraging Editas Medicine's unique platform technologies including Cas9 and AsCas12a. BMS has opted into six programs since the start of the collaboration, including one declared development candidate.

Corporate

- **Technology Platform**
  Expanded applications for SLEEK platform
  Last year, Editas Medicine announced the development of its SLEEK knock-in technology. The Company believes SLEEK will considerably change the development of gene edited engineered cell therapies, including iPSC-derived, healthy donor-derived, and autologously-derived therapies. More than 95% knock-in efficiencies have been demonstrated using the AsCas12a nuclease in various clinically relevant target cells, including iPSCs, T cells, and NK cells. Additionally, Editas Medicine believes SLEEK may be used to fine-tune the expression levels of transgene cargos, an important attribute of next-generation cell therapy medicines.

- **Leadership**
  Strengthened board with two additional directors in 2021
  Last 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 biopharma companies. Additionally, last October, the Company appointed Bernadette Connaughton to its Board of Directors and as Chairperson of the Company's Organization, Leadership and Compensation Committee. Ms. Connaughton is an accomplished pharmaceutical executive with more than 30 years of global strategic, commercial, and biopharmaceutical industry expertise.

- **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. Recent advancements will allow the Company to manufacture high quality guide ribonucleic acid (gRNA) to the appropriate scale, providing potential capital flexibility and lower costs associated with current supply chain infrastructure. The Company believes that these recent advancements will considerably enhance the research & development process.

Fourth Quarter and Full Year 2021 Financial Results

Cash, cash equivalents, and marketable securities as of December 31, 2021, were $619.9 million, compared to $657.0 million as of September 30, 2021, and $511.8 million as of December 31, 2020. The Company expects that its existing cash, cash equivalents and marketable securities will enable it to fund its operating expenses and capital expenditures through 2023.

Fourth Quarter 2021

- For the three months ended December 31, 2021, net loss attributable to common stockholders was $41.4 million, or $0.61 per share, compared to net loss of $62.5 million, or $1.00 per share, for the same period in 2020.

- Collaboration and other research and development revenues increased by $1.1 million to $12.5 million for the three months ended December 31, 2021, compared to $11.4 million for the same period in 2020. The increase was primarily attributable to additional programs licensed under our collaboration with Juno Therapeutics in 2021.

- Research and development expenses decreased by $23.9 million to $37.6 million for the three months ended December 31, 2021, from $61.5 million for the same period in 2020. The decrease related primarily to a recognition of $27.5 million in success payment expense related to a success payment triggering event in the three months ended December 31, 2020 and decreases in expenses in the three months ended December 31, 2021 as a result of our termination of our strategic alliance with Allergan in 2020, partially offset by increased manufacturing development of EDIT-301.

- General and administrative expenses increased by $0.7 million to $16.5 million for the three months ended December 31, 2021, from $15.8 million for the same period in 2020. The increase was primarily attributable to increased stock-based compensation and employee related expenses, partially offset by decreases in patent expense in the three months ended December 31, 2021.

Full Year 2021

- For the full year 2021, net loss attributable to common stockholders was $192.5 million, or $2.85 per share, compared to $116.0 million, or $1.98 per share, for the same period in 2020.

- Collaboration and other research and development revenues were $25.5 million for 2021, compared to $90.7 million for 2020. The $65.2 million decrease was primarily attributable to $70.6 million of revenue recognized in the year ended December 31, 2020 as a result of the termination of our strategic alliance with Allergan, partially offset by increases in revenue recognized in the year ended December 21, 2021 related to our collaboration agreement with Juno Therapeutics.

- Research and development expenses were $142.5 million for 2021, compared to $158.0 million for 2020. The $15.5 million decrease was primarily attributable to decreased costs in the year ended December 31, 2021 related to the termination of our strategic alliance agreements with Allergan and Sandhill in 2020, partially offset by increases in costs in the year ended December 31, 2021 related to manufacturing and clinical related costs, including costs to progress EDIT-101 and EDIT-301.

- General and administrative expenses were $76.2 million for 2021, compared to $67.6 million for 2020. The $8.6 million increase was primarily attributable to increased stock-based compensation expense related to the vesting of certain equity awards held by our former Chief Executive Officer in connection with her separation from the company in February 2021 as well as stock-based compensation expenses as a result of market-based and performance-based awards that were granted to our new Chief Executive Officer and certain other employees in 2021.

Upcoming Events

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

- American Associate for Cancer Research (AACR) Annual Meeting, April 8-13, New Orleans, LA
- The Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting, May 1-4, Denver, CO

Editas Medicine plans to participate in the following investor events:

- Cowen 42nd Annual Health Care Conference, March 8, 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 fourth quarter and full year 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 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 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 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 sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

EDIT-301 is currently being investigated in a clinical study in patients with severe SCD (RUBY trial, NCT04853576). Editas will initiate a new Phase 1/2 study of EDIT-301 in patients with TDT in 2022.

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).

About SLEEK Gene Editing
SLEEK (SeLection by Essential-gene Exon Knock-in) gene editing is an optimized approach to developing the next generation of cell therapy medicines for cancer and other serious diseases. Utilizing Editas Medicine's proprietary engineered AsCas12a nuclease, SLEEK enables high efficiency, multi-transgene knock-in of induced pluripotent stem cells (iPSCs), T cells, and natural killer (NK) cells while ensuring robust, transgene expression. Editas Medicine is currently leveraging SLEEK technology in its oncology programs.

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 first half of 2022, initiating dosing of the pediatric high-dose cohort in the BRILLIANCE trial 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, dosing the first TDT patient with EDIT-301 in 2022, and declaring an additional new development candidate for an *in vivo* ocular indication 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, 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.
Consolidated Statement of Operations
(amounts in thousands, except share and per share data)
(Unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
Collaboration and other research and development revenues	$ 12,469	$ 11,419	$ 25,544	$ 90,732
Operating expenses:				
Research and development	37,552	61,505	142,507	157,996
General and administrative	16,526	15,788	76,183	67,576
Total operating expenses	54,078	77,293	218,690	225,572
Operating loss	(41,609)	(65,874)	(193,146)	(134,840)
Other income, net:				
Other income(expense), net	(325)	2,853	(1,698)	16,259
Interest income, net	499	522	2,342	2,605
Total other income, net	174	3,375	644	18,864
Net loss	$ (41,436)	$ (62,499)	$ (192,502)	$ (115,976)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.61)	$ (1.00)	$ (2.85)	$ (1.98)
Weighted-average common shares outstanding, basic and diluted	68,355,723	62,278,035	67,619,388	58,609,389

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

	December 31, 2021	December 31, 2020
Cash, cash equivalents, and marketable securities	$ 619,915	$ 511,774
Working capital	460,426	360,879
Total assets	677,483	572,602
Deferred revenue, net of current portion	60,888	73,984
Total stockholders' equity	553,642	393,586

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