Editas Medicine Announces Fourth Quarter and Full Year 2020 Results and Update

February 25, 2021

Strengthened Leadership by Appointing James (Jim) C. Mullen as President and Chief Executive Officer, and Lisa A. Michaels, M.D., as Chief Medical Officer

Appointed Meeta Chatterjee, Ph.D., to Board of Directors

Initiated dosing of adult mid-dose cohort of BRILLIANCE trial of EDIT-101 for LCA10

Initiated Phase 1/2 RUBY trial for EDIT-301 for sickle cell disease

Ended 2020 with strong cash position of $512 million, and raised additional $250 million in early 2021

CAMBRIDGE, Mass., Feb. 25, 2021 (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 2020.

“Editas has a once-in-a-generation technology enabling us to develop transformational medicines. Entering 2021, we are advancing the landmark Brilliance trial, the first ever *in vivo* gene editing program and we look forward to reporting clinical data later this year,” said James C. Mullen, Chairman, President, and Chief Executive Officer, Editas Medicine. “Editas is also advancing our *ex vivo* gene edited medicines and we plan to begin enrollment in the Phase 1/2 RUBY clinical trial for the treatment of sickle cell disease. We also expect to make significant progress in our edited iNK cell therapy program for oncological treatments.”

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for Leber Congenital Amaurosis 10 (LCA10)**
  Dosed first patient in the adult mid-dose cohort of the BRILLIANCE trial
  Editas Medicine has dosed the first patient of the second cohort, the adult mid-dose, with EDIT-101. Initial clinical data is expected in 2021.

- **Retinitis Pigmentosa Type 4**
  On track for development candidate by end of 2021
  Editas Medicine is on-track to declare a development candidate for the treatment of Retinitis Pigmentosa Type 4 (adRP4) by the end of 2021. The Company plans to present preclinical data at a scientific meeting later this year.

***Ex Vivo* Gene Edited Cell Medicines**

- **EDIT-301 for Sickle Cell Disease**
  Initiation of Phase 1/2 RUBY trial
  The U.S. Food and Drug Administration (FDA) has approved the start of the Phase 1/2 RUBY study for sickle cell disease, and the Company can begin enrolling patients. Initial clinical sites for RUBY trial are expected to be approved by the second quarter of 2021, and the Company expects to dose the first patient before year-end. Editas Medicine is prepared for the manufacturing of clinical production of EDIT-301 through internal capabilities and manufacturing partners.

  On track for Investigational New Drug (IND) filing for the treatment of beta-thalassemia by end of 2021
  The Company is on track to file an IND application to the FDA for EDIT-301 for the treatment of beta-thalassemia by the end of 2021.

- **Edited iPSC NK (iNK) Cell Medicines to Treat Solid Tumors**
  Proprietary CRISPR/Cas12a editing of iPSCs enhances iNK tumor killing for potential off-the-shelf medicine
  Preclinical data presented at the 62nd Annual Meeting and Exposition of the American Society of Hematology (ASH) highlighted the Company’s CRISPR/Cas12a induced pluripotent stem cell (iPSC) editing platform. Editas Medicine demonstrated that knockout of *CISH* and *TGFβR2* with the proprietary CRISPR/Cas12a editing platform enhanced iNK cell tumor killing. Specifically, iNK cells containing the double knockout clones, were more effective than control iNK cells in killing tumor cells in a spheroid model. These data, supported by leveraged learnings from Editas Medicine’s discontinued healthy donor-derived NK cell program, continue to guide the Company’s clinical oncology strategy.

Corporate

- **Leadership**
  On February 15, 2021, James (Jim) Mullen was appointed as President and Chief Executive Officer of Editas Medicine, in addition to his existing role as Chairman of the Board. Jim is a recognized biotech leader with more than 40 years of experience building leading biotechnology and pharmaceutical organizations on a global scale. As CEO and President at Biogen, one of the world's largest biotechnology companies, Mr. Mullen oversaw the development and launch of many successful medicines. More recently, Mr. Mullen served as CEO of Patheon NV, a leading global provider of pharmaceutical development and manufacturing services, until its acquisition by Thermo Fisher Scientific in 2017.

  In November, the Company announced the appointment of Lisa A. Michaels, M.D., as Chief Medical Officer. Dr. Michaels has more than 25 years of experience in clinical research and drug development in both industry and academia. Dr. Michaels joined Editas Medicine from Bayer Pharmaceuticals where she spent more than 10 years in drug development, leading teams from early research and drug discovery through regulatory approval, commercial launch, and life cycle management. Most recently, she served as head of Bayer's Rare Diseases, Cell & Gene Therapy therapeutic area.

  In December, the Company appointed Meeta Chatterjee, Ph.D., to its Board of Directors. Dr. Chatterjee is an accomplished biopharmaceutical executive with more than 30 years of broad strategic and operational experience in research and development, mergers and acquisition evaluation, in-licensing, and externalization activities.

- **Manufacturing**
  Editas Medicine continues to advance internal and external manufacturing capabilities for the Company's portfolio of *in vivo* gene edited medicines and *ex vivo* gene edited cell medicines. The transfer of manufacturing materials from AbbVie to Editas Medicines was completed and the Company can progress the ocular medicines pipeline following reacquiring the rights from AbbVie. The Company is also expanding internal capability of manufacturing GMP guide RNA's for future programs at the Boulder, Colorado facility.

- **Balance Sheet**
  The Company expects that its existing cash, cash equivalents and marketable securities of $512 million at December 31, 2020, will enable it to fund its operating expenses and capital expenditures into 2023. This cash position does not include the $250 million of net proceeds from the public offering that occurred in early 2021.

Fourth Quarter and Full Year 2020 Financial Results

Cash, cash equivalents, and marketable securities at December 31, 2020, were $512 million, compared to $541 million at September 30, 2020, and $457 million at December 31, 2019.

For the three months ended December 31, 2020, net loss was $62.5 million, or $1.00 per share, compared to $37.8 million, or $0.74 per share, for the same period in 2019.

- Collaboration and other research and development revenues decreased by $0.9 million, to $11.4 million for the three months ended December 31, 2020 from $12.3 million for three months ended December 31, 2019. This decrease was primarily attributable to the recognition of revenue under the Allergan collaboration in the three months ended December 31, 2019 for which there was no similar revenue in the three months ended December 31, 2020 as a result of the termination of our strategic alliance with Allergan.

- Research and development expenses increased by $26.7 million, to $61.5 million for the three months ended December 31, 2020 from $34.8 million for the three months ended December 31, 2019. The $26.7 million increase was primarily attributable 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 an increase in expenses related to the clinical and manufacturing development of EDIT-101, EDIT-301 and our other programs, partially offset by a decrease in sublicensing expense.

- General and administrative expenses decreased by $1.1 million to $15.8 million for the three months ended December 31, 2020, from $16.9 million for the same period in 2019. The $1.1 million decrease was primarily attributable to a decrease in expense related to stock-based compensation due to a modification that occurred in 2019 for which there was no similar activity in 2020 and decreased performance bonus expense due to employee turnover in the fourth quarter of 2020.

For the full year 2020, net loss attributable to common stockholders was $116.0 million, or $1.98 per share, compared to $133.7 million, or $2.68 per share, for the same period in 2019.

- Collaboration and other research and development revenues were $90.7 million for 2020, compared to $20.5 million for 2019. The $70.2 million increase was primarily attributable to a $57.1 million increase in the revenue recognized as a result of the termination of our strategic alliance with Allergan as well as increases in revenue recognized pursuant to our

other collaboration and out-licensing agreements.

- Research and development expenses were $158.0 million for 2020, compared to $96.9 million for 2019. The $61.1 million increase was primarily attributable to increased process and platform development expenses driven by increased manufacturing and clinical related costs, including costs to progress EDIT-101 and EDIT-301, increased expenses related to the triggering of a $27.5 million success payment in 2020 and increased employee-related costs, partially offset by a decrease in sublicense and license fees. Remaining success payments will only be payable upon a combination of product candidate and market capitalization triggers.

- General and administrative expenses were $67.6 million for 2020, compared to $64.6 million for 2019. The $3.0 million increase was primarily attributable to increased employee-related costs and facility expenses due to an increased workforce, partially offset by decreased stock-based compensation expense.

Upcoming Events

Editas Medicine will participate in the following investor events:

- Cowen & Company 41st Annual Health Care Conference, March 1, Virtual
- Barclays Global Healthcare Conference, March 10, Virtual

Editas Medicine will participate in the following scientific and medical conferences:

- American Association of Cancer Research Annual Meeting, April 10-15, Virtual
- Association for Research in Vision and Ophthalmology Annual Meeting, May 1-7, 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 2020. To access the call, please dial (844) 348-3801 (domestic) or (213) 358-0955 (international) and provide the passcode 7172199. A live webcast of the call will be available on the Investors & Media 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 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 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.

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 the initiation of the RUBY trial and filing an IND for EDIT-301 for the treatment of beta-thalassemia by the end of 2021, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including EDIT-101, and the timing or likelihood of regulatory filings and approvals. 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. These and other risks are described in greater detail under the caption "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 speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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,	
	2020	2019	2020	2019
Collaboration and other research and development revenues	$ 11,419	$ 12,284	$ 90,732	$ 20,531
Operating expenses:				
Research and development	61,505	34,789	157,996	96,898
General and administrative	15,788	16,918	67,576	64,555
Total operating expenses	77,293	51,707	225,572	161,453
Operating loss	(65,874)	(39,423)	(134,840)	(140,922)
Other income, net:				
Other income(expense), net	2,853	8	16,259	(137)
Interest income, net	522	1,645	2,605	7,313
Total other income, net	3,375	1,653	18,864	7,176
Net loss	$ (62,499)	$ (37,770)	$ (115,976)	$ (133,746)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.00)	$ (0.74)	$ (1.98)	$ (2.68)
Weighted-average common shares outstanding, basic and diluted	62,278,035	51,169,242	58,609,389	49,983,329

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	$ 511,774	$ 457,140
Working capital	358,894	403,881
Total assets	572,602	508,885
Deferred revenue, net of current portion	73,984	163,207
Total stockholders' equity	393,586	262,437

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