Editas Medicine Announces Fourth Quarter and Full Year 2019 Results and Update

February 26, 2020

Announcement of first patient dosing with EDIT-101 (AGN-151587) expected in 1Q20

Plan to file IND for EDIT-301 for sickle cell disease by end of 2020

Research collaboration with Sandhill Therapeutics accelerates IND-enabling studies for allogeneic healthy donor NK program to treat solid tumors in mid-2020

CAMBRIDGE, Mass., Feb. 26, 2020 (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 2019.

"We are entering 2020 with strong momentum and a strategic focus on driving our pipeline of *in vivo* CRISPR and engineered cell medicines forward with the ultimate vision of developing differentiated, transformational medicines for people living with serious diseases," said Cynthia Collins, Chief Executive Officer of Editas Medicine. "Our team is making history with the first ever clinical trial of an *in vivo* CRISPR medicine, advancing our broader pipeline of *in vivo* CRISPR medicines, and progressing our engineered cell medicines for hemoglobinopathies and cancers. With our recent achievements, I expect our clinical pipeline to yield a robust and sustainable portfolio of differentiated, transformative medicines and ensure the Company's long-term growth."

Recent Achievements and Outlook

***In Vivo* CRISPR Medicines**

- **EDIT-101 (AGN-151587) for LCA10**
  First in vivo *CRISPR gene editing trial initiated*
  Editas Medicine (Company) and its partner, Allergan, are conducting the Brilliance Phase 1/2 clinical trial to evaluate the safety, tolerability, and efficacy of EDIT-101 as a treatment for Leber congenital amaurosis 10 (LCA10). An announcement of first patient dosing is expected in the first quarter of 2020.

- **EDIT-102 for Usher Syndrome 2A**
  Ready for IND-enabling studies
  EDIT-102 is designed to remove exon 13 of the *USH2A* gene to restore functional Usherin protein using the same proprietary *S. aureus* Cas9 enzyme, AAV vector, and tissue-specific promoter as EDIT-101. Under the terms of its alliance agreement with Allergan, the Company has delivered a preclinical data package for EDIT-102 to Allergan for potential licensing and initiation of Investigational New Drug (IND)-enabling studies.

Engineered Cell Medicines

- **EDIT-301 for Sickle Cell Disease and Beta-Thalassemia**
  IND filing for Sickle Cell Disease targeted by end of 2020
  Editas Medicine is developing EDIT-301 using Cas12a (Cpf1) as a potentially best-in-class medicine to treat sickle cell disease and beta-thalassemia. Preclinical *in vivo* data were shared at the 61st Annual Meeting of the American Society of Hematology and IND-enabling studies are in progress.

- **Oncology**
  Plan to initiate IND-enabling studies for allogeneic natural killer (NK) cell medicine in mid-2020
  Editas Medicine has formed a strategic research collaboration with Sandhill Therapeutics, Inc., to develop allogeneic healthy donor derived NK cell medicines for the treatment of solid tumors. As a result, the Company expects to initiate IND-enabling studies for an experimental engineered oncology medicine to treat solid tumors in mid-2020. This approach complements Editas Medicine's programs developing universal donor NK cell medicines derived from induced pluripotent stem cells.

Corporate

- **Leadership**
  The Company has strengthened its executive leadership team with the appointments of Michelle Robertson as Chief Financial Officer and Harry Gill as Senior Vice President, Operations. Ms. Robertson joins Editas Medicine with more than 25 years of finance and commercial operations management experience in biotechnology companies. Mr. Gill brings more than 30 years of life sciences experience with leadership roles in quality, plant operations, technical services, and

operational excellence.

- **Intellectual Property**
  On June 24, 2019, the U.S. Patent and Trademark Office declared an interference between certain CRISPR/Cas9 patent applications submitted by the University of California, the University of Vienna, and Emmanuelle Charpentier and certain patents issued to the Broad Institute, Inc. (Broad) that have been licensed to Editas Medicine. The Broad patents remain valid and in force. Foundational claims covering the use of CRISPR/Cas9 for gene editing in eukaryotic cells have issued and continue to issue to Broad as patents in the United States, Europe, Japan, and other jurisdictions.

- **Balance Sheet**
  The Company expects that its existing cash, cash equivalents and marketable securities of $457.1 million at December 31, 2019, will enable it to fund its operating expenses and capital expenditures for at least the next 24 months.

Upcoming Events

Editas Medicine will participate in the following investor events:

- Cowen & Company 40th Annual Health Care Conference, March 4, 10:40 a.m. ET, Boston; and
- Barclays Global Healthcare Conference, March 10, 10:15 a.m. ET, Miami.

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

- American Association for Cancer Research Annual Meeting 2020, April 24-29, San Diego;
- Association for Research in Vision & Ophthalmology 2020, May 3-7, Baltimore; and
- 23rd Annual Meeting of the American Society of Gene & Cell Therapy, May 12-15, Boston.

Fourth Quarter and Full Year 2019 Financial Results

Cash, cash equivalents, and marketable securities at December 31, 2019, were $457.1 million, compared to $332.6 million at September 30, 2019, and $369.0 million at December 31, 2018.

For the three months ended December 31, 2019, net loss was $37.8 million, or $0.74 per share, compared to $25.1 million, or $0.52 per share, for the same period in 2018.

- Collaboration and other research and development revenues were $12.3 million for the three months ended December 31, 2019, compared to $6.1 million for the same period in 2018. The $6.2 million increase was primarily attributable to $5.0 million in increased revenue recognized pursuant to our amended collaboration agreement with Celgene and $1.1 million in increased revenue recognized pursuant to our strategic alliance with Allergan.

- Research and development expenses increased by $15.6 million, to $34.8 million for the three months ended December 31, 2019, from $19.2 million for the same period in 2018. The $15.6 million increase was primarily attributable to increased process and platform development expenses driven by increased manufacturing and clinical related costs, including costs under our profit-sharing arrangement with Allergan in the United States for EDIT-101 and increased sublicensing payment expenses owed to certain of our licensors in connection with the amended collaboration agreement with Celgene.

- General and administrative expenses increased by $3.7 million to $16.9 million for the three months ended December 31, 2019, from $13.2 million for the same period in 2018. The $3.7 million increase was primarily attributable to increased professional service expenses.

For the full year 2019, net loss was $133.7 million, or $2.68 per share, compared to $110.0 million, or $2.33 per share, for the same period in 2018.

- Collaboration and other research and development revenues were $20.5 million for 2019, compared to $31.9 million for 2018. The $11.4 million decrease was attributable to $15.0 million in revenue recognized during the third quarter of 2018 related to the EDIT-101 option exercise payment pursuant to our strategic alliance with Allergan and $3.9 million in revenue recognized during the second quarter of 2018 related to a one time upfront payment in connection with an out-license arrangement.

- Research and development expenses were $96.9 million for 2019, compared to $90.7 million for 2018. The $6.2 million increase was attributable to increased process and platform development expenses driven by increased manufacturing and clinical related costs, including costs under our profit-sharing arrangement with Allergan in the United States for EDIT-101, and increased employee-related costs, partially offset by a decrease in success payment expenses.

- General and administrative expenses were $64.6 million for 2019, compared to $55.0 million for 2018. The $9.6 million increase was primarily attributable to increased professional service expenses.

Conference Call

The Editas Medicine management team will host a conference call and webcast today at 5:00 p.m. ET to provide and discuss a corporate update and financial results for the fourth quarter and full year 2019. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 1609775. 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 the Editas Medicine-Allergan Alliance

In March 2017, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) entered a strategic alliance and option agreement under which Allergan received exclusive access and the option to license up to five of Editas Medicine's genome editing programs for ocular diseases, including EDIT-101 (AGN-151587). Under the terms of the agreement, Allergan is responsible for development and commercialization of optioned products, subject to Editas Medicine's option to co-develop and share equally in the profits and losses of two optioned products in the United States. In August 2018, Allergan exercised its option to develop and commercialize EDIT-101 globally for the treatment of LCA10. Additionally, Editas Medicine exercised its option to co-develop and share equally in the profits and losses from EDIT-101 in the United States. Editas Medicine is also eligible to receive development and commercial milestones, as well as royalty payments on a per-program basis. The agreement covers a range of first-in-class ocular programs targeting serious, vision-threatening diseases based on Editas Medicine's unparalleled CRISPR genome editing platform, including CRISPR/Cas9 and CRISPR/Cas12a (also known as Cpf1).

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 Company's plans with respect to the Brilliance Phase 1/2 clinical trial for EDIT-101 (AGN-151587), including expecting an announcement of dosing in Q1 2020, filing an IND for EDIT-301 by the end of the year and initiating IND-enabling studies for an experimental medicine to treat solid tumors in mid-2020. 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 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 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,	
	2019	2018	2019	2018
Collaboration and other research and development revenues	$ 12,284	$ 6,119	$ 20,531	$ 31,937
Operating expenses:				
Research and development	34,789	19,195	96,898	90,654
General and administrative	16,917	13,177	64,555	55,010
Total operating expenses	51,706	32,372	161,453	145,664
Operating loss	(39,422)	(26,253)	(140,922)	(113,727)
Other income, net:				
Other income(expense), net	8	(3)	(137)	328
Interest income, net	1,645	1,202	7,313	3,445
Total other income, net	1,653	1,199	7,176	3,773
Net loss	$ (37,770)	$ (25,054)	$ (133,746)	$ (109,954)

Net loss per share attributable to common stockholders, basic and diluted	$ (0.74)	$ (0.52)	$ (2.68)	$ (2.33)
Weighted-average common shares outstanding, basic and diluted	51,169,242	48,006,980	49,983,329	47,097,735

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

	December 31, 2019	December 31, 2018
Cash, cash equivalents, and marketable securities	$ 457,140	$ 368,955
Working capital	403,881	338,876
Total assets	508,885	420,386
Deferred revenue, net of current portion	163,207	115,614
Construction financing lease obligation, net of current portion	----------	32,417
Total stockholders' equity	262,437	236,162

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