Editas Medicine Reports on Recent Progress and Outlook at J.P. Morgan Healthcare Conference

January 10, 2022

Anticipated 2022 milestones include initial clinical data for EDIT-301 in sickle cell disease by year-end, dosing of first TDT patient with EDIT-301, initiation of pediatric high-dose cohort for EDIT-101 in LCA10, and a clinical data update on EDIT-101 in the second half of 2022

Company announces new development candidates EDIT-103 for RHO-adRP and EDIT-202 multiplexed iNK cell therapy for solid tumors

CAMBRIDGE, Mass., Jan. 10, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced that James C. Mullen, Chairman, President, and Chief Executive Officer, will discuss the Company's recent progress and outlook for its gene editing medicines and platform technology at the 40th Annual J.P. Morgan Healthcare Conference being held virtually on Wednesday, January 12 at 10:30 a.m. EST.

"Editas is developing a high-value pipeline of gene editing therapeutic candidates enabled by best-in-class next generation technologies, such as our efficient, high precision AsCas12a nuclease and SLEEK editing platform," said Mr. Mullen. "In 2021, we achieved proof-of-concept with EDIT-101 in LCA10, which supports our foundational *in vivo* technology in additional ocular indications, such as our new development candidate EDIT-103 for RHO-adRP. This year we expect to obtain initial clinical results from our *ex vivo* EDIT-301 program in sickle cell disease and dose the first patient in the EDIT-301 study of transfusion-dependent beta thalassemia. We're also excited to advance our alpha-beta T cell oncology program, partnered with Bristol Myers Squibb, and our EDIT-202 iNK cell therapy for solid tumors towards the clinic."

Recent Progress and Upcoming Milestones

***In Vivo* Gene Edited Medicines Outlook**

- 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 following a safety assessment by an independent data monitoring committee.
    - Additionally, the Company is expanding enrollment in one or more of the previously completed adult cohorts to support anticipated registrational trial design and endpoints. Editas will provide additional details regarding the expansion later this year.
    - Editas also 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 6 months of follow-up evaluations.
    - The Company also anticipates establishing registrational trial criteria for EDIT-101 by year-end 2022.
- The Company is advancing additional *in vivo* ocular editing programs, leveraging its learnings from EDIT-101, and has declared a new development candidate, EDIT-103, for treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP), a progressive form of retinal degeneration, which is progressing towards IND-enabling studies. EDIT-103 uses two adeno-associated virus (AAV) vectors to knockout and replace mutations in the rhodopsin gene in order to preserve photoreceptor function. Editas Medicine believes that simultaneously knocking out and replacing a gene in the same cell *in vivo* is a significant technical breakthrough, potentially leading to findings that could address other autosomal dominant diseases, where gain of negative function needs to corrected.
- 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 a AsCas12a nuclease, Editas Medicine has enhanced productive editing by approximately 350% compared to the initial construct.
- The Company expects to declare an additional new development candidate for an *in vivo* ocular indication in 2022.

***Ex Vivo* Gene Edited Medicines Outlook**

- The Company is developing EDIT-301 for the treatment of sickle cell disease and transfusion-dependent beta thalassemia (TDT). Following natural human genetics, Editas is editing the HBG1/2 promoter mimicking naturally occurring human mutations, analogous to patients with Hereditary Persistence of Fetal Hemoglobin (HPFH), ameliorating disease symptoms. Using Editas Medicine's proprietary engineered AsCas12a enzyme, which has demonstrated high efficiency editing of multipotent long-term hematopoietic stem cells for sustained efficacy and durability, EDIT-301 was specifically designed to generate these highly reproducible edits of CD34+ cells. 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 the end of 2022.

- The Company recently received clearance from the U.S. Food and Drug Administration of its investigational new drug application for EDIT-301 for the treatment of TDT, and expects to initiate dosing in a Phase 1/2 clinical study in TDT during 2022.

Cellular Therapy Outlook

- 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, as demonstrated by recently presented preclinical data. Editas Medicine expects to further optimize and advance this therapy in preclinical development during 2022.
- 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 Cas12a. BMS has opted into six programs since the start of the collaboration, including one declared development candidate.
- Editas Medicine continues to evaluate the application of its iPSC platform to additional cell types beyond iNK and alpha-beta T cells. These include gamma-delta T cells for oncology indications and multiple cell types for regenerative medicine applications of iPSCs.

Corporate Strategy Updates

- Editas Medicine has undertaken a strategic review to inform new development opportunities to which it can apply its industry-leading gene editing technologies including the SLEEK platform and its proprietary engineered AsCas12a nuclease. The Company expects to expand applications of its gene editing platform, including additional ocular diseases, *in vivo* applications, regenerative medicine applications, and oncology indications. Additionally, the Company is advancing technology to improve gene editing and delivery modalities.
- The Company plans to pursue future development and commercialization opportunities in areas outside of its core strategic focus through partnerships. Potential future partnerships include oncology indications with its cell therapy platform, regenerative medicine applications of its iPSC technology, and international expansion. In addition, Editas may out-license select applications of its proprietary technologies such as Cas9, AsCas12a, and SLEEK.

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.

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.

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 by year end 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, 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 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 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.

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A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/9e875306-7c1c-4929-8e4c-f98ea38307ea

Source: Editas Medicine, Inc.

James C. Mullen

James C. Mullen, Chairman, President, and Chief Executive Officer of Editas Medicine