Editas Medicine Presents Data on SLEEK Gene Editing Technology at the American Society of Gene and Cell Therapy Annual Meeting

May 18, 2022

SLEEK results in highly efficient multi-transgene knock-in and enables tunability of transgene expression

Double knock-in iNK cells demonstrate strong tumor killing effect and prolonged in vivo persistence in an ovarian cancer mouse model

CAMBRIDGE, Mass., May 18, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, will present data later today on its SLEEK (SeLection by Essential- gene Exon Knock-in) gene editing technology during the New Gene Editing Technologies and Applications Session at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) being held in Washington, D.C., and virtually, May 16 – 19, 2022.

“Despite recent progress, many cell-based medicines suffer from inefficient knock-in of transgene cargos and high heterogeneity that may reduce efficacy and pose potential safety issues. SLEEK is designed to address these challenges, by achieving efficient knock-in of multiple transgenes, avoiding silencing of transgene cargos over time, and enabling efficient knock-in using non-viral DNA donor templates,” said Mark S. Shearman, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. “We have used SLEEK to achieve very high knock-in efficiencies in T cells, B cells and natural killer (NK) cells. Our SLEEK-edited, iPSC-derived double knock-in NK cells resulted in impressive tumor clearance, improved survival, and prolonged *in vivo* persistence in an ovarian cancer mouse model. We believe this technology has the potential to fundamentally improve the next generation of cell-based medicines, beginning with EDIT-202, our first iPSC-derived NK cell oncology program now in preclinical studies.”

SLEEK was used to insert CD16 and mbIL-15 cargos into iPSCs which generated NK cells with significantly improved tumor killing and survival compared to wild-type NK cells. SLEEK-edited cells also demonstrated strong antibody-dependent cellular cytotoxicity and improved persistence, without the need for exogenous cytokine support.

Further key findings presented about SLEEK include:

- Enables knock-in editing efficiencies of over 90% in relevant cell types, without impacting long-term viability or expansion.
- Permits constitutive and robust expression in iPSC-derived cell types where transgene promoter silencing has been a major challenge during the differentiation process.
- Demonstrated utility with clinically important cargos including chimeric antigen receptors (CARs) and allogeneic shields.
- Provides targeted knock-in to clinically important ex vivo cell types including T cells, B cells, and NK cells.
- Allows for tunability of transgene expression based on different promoter strengths of various essential genes.
- Enables use of non-viral DNA templates matching performance of the AAV6 gold standard on key attributes, potentially supporting more complex knock-in cassettes by avoiding AA6 cargo capacity limits.

Additional Presentations at ASGCT
In an oral presentation at the ASGCT Annual Meeting on Monday, May 16, 2022, Editas presented data on its EDIT-101 program for the treatment for Leber Congenital Amaurosis 10 (LCA10), a CEP290-related retinal degenerative disorder, demonstrating a favorable immunogenicity profile.

On Thursday, May 19, 2022, Editas will provide an oral presentation on its EDIT-103 program, in development for the treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP). The presentation will highlight preclinical data from non-human primate studies of EDIT-103, demonstrating nearly 100% gene editing knockout of endogenous RHO gene and more than 30% replacement protein levels.

The complete list of Editas Medicine presentations at ASGCT is below. Abstracts can be accessed on the ASGCT website, and the presentations will be posted on the Editas Medicine website during the conference.

Oral Presentations:
Title: Exploratory Immuno-Safety Profile of EDIT-101, a First-in-Human *In Vivo* CRISPR Gene Editing Therapy for *CEP290*-Related Retinal Degeneration
Session Date and Time: Monday, May 16, 2022, 1:30 p.m. – 3:15 p.m. ET
Presentation Time: 2:45 p.m. – 3:00 p.m. ET
Session title: Gene and Cell Therapy Trials in Progress

Title: SLEEK: A Method for Highly Efficient Knock-in and Expression of Transgene Cargos for Next-generation Cell-based Medicines
Session Date and Time: Wednesday, May 18, 2022, 3:45 p.m. – 5:30 p.m. ET
Presentation Time: 5:00 p.m. – 5:15 p.m. ET
Session title: New Gene Editing Technologies and Applications

Title: A Mutation-Independent CRISPR/Cas9-Based ‘Knockout and Replace’ Strategy to Treat Rhodopsin-Associated Autosomal Dominant Retinitis Pigmentosa
Session Date and Time: Thursday May 19, 2022, 10:15 a.m. – 12:00 p.m. ET
Presentation Time: 10:15 a.m. – 10:30 a.m. ET
Session title: Ophthalmic and Auditory Diseases

Poster Presentation:
Title: EDIT-202, A Multiplexed CRISPR-Cas12a Gene-Edited iPSC-Derived NK Cell Therapy has Prolonged Persistence, Promotes High Cytotoxicity, and Enhances *In Vivo* Tumor Killing
Session Date and Time: Wednesday, May 18, 2022, 5:30 p.m. – 6:30 p.m. ET
Session Title: Cancer - Targeted Gene and Cell Therapy II

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 (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.

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. 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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