



Editas Medicine to Present New Data Demonstrating Progress Towards Creating Genome Editing Medicines at the American Society of Gene & Cell Therapy Annual Meeting

April 30, 2018

Oral presentation of tolerability and immunogenicity data from a study of EDIT-101 following subretinal injection in non-human primates

CAMBRIDGE, Mass., April 30, 2018 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced that 10 scientific abstracts, including three from research collaborations, have been accepted for presentation at the 21st Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT). The meeting will take place May 16-19 in Chicago. The Company is presenting data on its pipeline and platform technologies to support ongoing development programs.

Key Editas Medicine presentations at ASGCT will include data demonstrating:

- EDIT-101, the Company's experimental medicine for the treatment of Leber Congenital Amaurosis type 10, administered to non-human primates via subretinal injection was well-tolerated;
- Therapeutically relevant editing levels in non-human primates regardless of pre-existing or induced immunity to *Staphylococcus aureus* Cas9;
- Specificity of EDIT-101 with no verified off-targets in the human genome as assessed by a two-staged approach using orthogonal methods;
- *In vitro* validation of an exon deletion editing strategy with the potential to treat Usher Syndrome type 2a (USH2A)-associated retinal disease;
- *In vivo* proof-of-concept in animal models for developing a CRISPR-based medicine for the treatment of ocular herpetic keratitis caused by latent herpes simplex virus-1 (HSV-1); and
- Fetal hemoglobin induction by editing of novel therapeutic sites identified through saturation genomic CRISPR screening of the beta-globin locus as a potential treatment for sickle cell anemia.

In addition, Editas Medicine's Chief Scientific Officer will speak at the ASGCT Gene Editing Workshop, a pre-meeting program, on May 15.

"We are making significant scientific advances in our programs to unlock the potential of CRISPR genome editing for making medicines," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "At ASGCT, we will showcase our recent scientific advances as we make progress towards the clinic and towards our goal of making medicines for people living with serious diseases."

The complete list of Editas Medicine presentations is below. Abstracts can be accessed on the ASGCT website at <https://plan.core-apps.com/asgct2018/abstracts>.

Oral Presentation:

[Evaluation of Tolerability and Immunogenicity of EDIT-101 Following Subretinal Injection in Non-human Primate](#)

Date/Time: May 18, 4:00 – 4:15 p.m.

Location: Salon A-5

Session: Preclinical Pharmacology and Toxicology Studies and Assessment of Gene Therapy in Large Animal Models

Editas Medicine Poster Presentations:

[Treatment of Herpetic Keratitis with CRISPR/Cas9 Gene Editing in a Rabbit Disease Model](#)

Date/Time: May 16, 5:30 – 7:30 p.m.

Location: Stevens Salon C, D

Session: Neurologic Diseases (Including Ophthalmic and Auditory Diseases) I

[Potent HbF Induction Following ssODN-Mediated Repair of Cas9-Induced DSB at the HBG Promoter in CD34+ HSPC](#)

Date/Time: May 16, 5:30 – 7:30 p.m.

Location: Stevens Salon C, D

Session: Hematologic & Immunologic Diseases I

[Saturated Mutagenesis Surrounding Beta-globin Locus Identifies Novel Therapeutic Targets for Fetal Globin Induction and Treatment of Sickle Cell Anemia](#)

Date/Time: May 16, 5:30 – 7:30 p.m.

Location: Stevens Salon C, D

Session: Hematologic & Immunologic Diseases I

[Improving Efficacy of CAR T cells Through CRISPR/Cas9 Mediated Knockout of TGF \$\beta\$ R2](#)

Date/Time: May 16, 5:30 – 7:30 p.m.

Location: Stevens Salon C, D

Session: Cancer – Targeted Gene & Cell Therapy I

[Efficient Targeted Integration in Human T cells with CRISPR-Cas9 for the Treatment of X-Linked Hyper-IgM Syndrome](#)

Date/Time: May 17, 5:15 – 7:15 p.m.

Location: Stevens Salon C, D

Session: Hematologic & Immunologic Diseases II

[Gene Editing Specificity Assessment for EDIT-101, an LCA10 Therapeutic Candidate](#)

Date/Time: May 18, 5:45 – 7:45 p.m.

Location: Stevens Salon C, D

Session: Pharmacology/Toxicology Studies or Assay Development

Research Collaboration Poster Presentations (Editas Medicine Author):

[Development of an Assay to Detect Pre-existing Anti-Cas9 Antibodies and an Estimate of the Prevalence of Anti-Staphylococcus- and Streptococcus-Cas9 Antibodies in the US Population](#)

Date/Time: May 17, 5:15 – 7:15 p.m.

Location: Stevens Salon C, D

Session: Gene Targeting & Gene Correction II

[Preclinical Modeling Highlights the Therapeutic Potential of the Adoptive Transplant of Gene Corrected T cells in X-Linked Hyper-IgM Syndrome](#)

Date/Time: May 18, 5:45 – 7:45 p.m.

Location: Stevens Salon C, D

Session: Hematologic & Immunologic Diseases

[Evaluation of Therapeutic Potential of Human USH2A Gene Lacking Exon 13 \(USH2A-ΔEx13\) for Restoring Ciliogenesis](#)

Date/Time: May 18, 5:45 – 7:45 p.m.

Location: Stevens Salon C, D

Session: Neurologic Diseases (Including Ophthalmic and Auditory Diseases) I

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/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, 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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