Editas Medicine Presents Preclinical Data on EDIT-103 for Rhodopsin-associated Autosomal Dominant Retinitis Pigmentosa at the Association for Research in Vison and Ophthalmology Conference

May 4, 2022

Studies in non-human primates demonstrated nearly 100% gene editing knockout of endogenous RHO gene and more than 30% replacement protein levels

Treated eyes showed morphological and functional photoreceptor preservation

EDIT-103 advancing towards IND-enabling studies

CAMBRIDGE, Mass., May 04, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced *ex vivo* and *in vivo* preclinical data for its experimental medicine EDIT-103 for the treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP). The Company reported these data in an oral presentation today at the Association for Research in Vison and Ophthalmology (ARVO) Annual Meeting in Denver.

EDIT-103 is a mutation-independent CRISPR/Cas9-based, dual AAV5 vectors “knockout and replace” (KO&R) therapy to treat RHO-adRP. This approach has the potential to treat any of over 150 dominant gain-of-function rhodopsin mutations that cause RHO-adRP with a one-time subretinal administration.

“These promising data in non-human primates and mice demonstrate the potential of EDIT-103 to efficiently remove the defective RHO gene responsible for RHO-adRP, and, critically, replace it with a sufficient level of RHO to preserve photoreceptor structure and functions,” said Mark S. Shearman, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. “The program is progressing toward the clinic, and we expect to start IND-enabling studies by the end of 2022. EDIT-103, which uses a dual AAV gene editing approach, also provides initial proof of concept for the treatment of other autosomal dominant disease indications where a gain of negative function needs to be corrected, and we are evaluating the development of additional programs.”

Key findings include:

- In human retina explants, EDIT-103 demonstrated highly specific editing with no off-target editing observed after transduction.
- In a *mRho*$^{hRHO/+}$ mouse model, EDIT-103 achieved rapid gene editing, with maximal levels at six (6) weeks and sustained, stable editing until end of study (13 weeks).
- In non-human primates (NHPs), EDIT-103 demonstrated nearly 100% knockout of endogenous RHO, and the replacement RHO gene produced over 30% of normal RHO protein levels in the treated area of subretinal injection.
- The EDIT-103 (KO&R)-injected eyes of NHPs showed restoration of RHO expression in the outer segments and retention of normal photoreceptor structure and function compared to the KO-injected eye.

Presentation Session Information:

Presentation Title: A Mutation-Independent CRISPR/Cas9-based ‘Knockout and Replace’ Strategy to Treat Rhodopsin-Associated Autosomal Dominant Retinitis Pigmentosa

Session Title: Gene therapy and other novel therapeutics in ophthalmic diseases 2

Session Date and Time: May 4, 2022, from 10:00 a.m. to 12:00 p.m. MDT

Presentation Time: May 4, 2022, 11:08 a.m. MDT (1:08 p.m. EDT)

Presenter: Zoe (Chi-Hsiu) Liu, Senior Scientist I, *In Vivo* Gene Editing, Editas Medicine

Location: Denver Convention Center, 4AB Mile High Ballroom

Full details of the Editas Medicine presentations can be accessed in the **Posters & Presentations** section on the Company’s website.

About EDIT-103

EDIT-103 is a CRISPR/Cas9-based experimental medicine in preclinical development for the treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP), a progressive form of retinal degeneration. EDIT-103 is administered via subretinal injection and uses two adeno-associated virus (AAV) vectors to knockout and replace mutations in the rhodopsin gene to preserve photoreceptor function. This approach can potentially address more than 150 gene mutations that cause RHO-adRP.

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 Company's expectation to start IND-enabling studies for EDIT-103 by the end of 2022. 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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