Allergan and Editas Medicine Announce Initial Data from Natural History Study to Evaluate Patients with Leber Congenital Amaurosis 10 (LCA10)

April 29, 2019

DUBLIN, Ireland and CAMBRIDGE, Mass., April 29, 2019 (GLOBE NEWSWIRE) -- Allergan plc (NYSE: AGN), a leading global pharmaceutical company, and Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, announced initial data from the ongoing natural history study to evaluate patients with Leber congenital amaurosis 10 (LCA10), a rare form of blindness caused by mutations in the *CEP290* gene. The Companies reported these data on Friday, April 26, in an oral presentation at the 6th Annual Retinal Cell and Gene Therapy (RCGT) Innovation Summit in Vancouver.

The Companies initiated the natural history study to prospectively evaluate and confirm the course of disease in patients and determine the reproducibility and stability of ophthalmic assessments over time. This knowledge has informed the planned Phase 1/2 interventional clinical trial design for AGN-151587 (EDIT-101), an experimental CRISPR genome editing medicine being investigated for the treatment of LCA10. Massachusetts Eye and Ear, an international center for treatment and research and a teaching hospital of Harvard Medical School, is one of seven sites enrolling patients in this natural history study.

“Better understanding the course of disease for patients with CEP290-associated retinal degeneration is a key step towards the interventional clinical trial for a CRISPR-based experimental medicine to treat this disease,” said Eric A. Pierce, M.D., Ph.D., Director of the Ocular Genomics Institute and William F. Chatlos Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School, and Principal Investigator for the natural history study. “We are pleased to be working at the forefront of this research with Allergan and Editas Medicine and look forward to testing genome editing for the treatment of CEP290-associated retinal disease in the interventional trial.”

Twenty-one patients with LCA10-IVS26 mutations in CEP290 were included in this analysis of baseline characteristics and key clinical assessments, including visual acuity and full-field threshold sensitivity changes. Initial data from the natural history study suggest multiple clinical assessments should prove informative in the planned Phase 1/2 interventional trial.

AGN-151587 is set to be the first *in vivo,* or inside the body, CRISPR-based medicine administered to people anywhere in the world. In the planned Phase 1/2 clinical trial, Allergan and Editas Medicine intend to initiate patient screening mid-year and begin patient dosing in the second half of 2019, enrolling 10-20 patients in the U.S. and Europe.

About AGN-151587 (EDIT-101)
AGN-151587 (EDIT-101) is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10). AGN-151587 is administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells.

About Leber Congenital Amaurosis
Leber congenital amaurosis, or LCA, is a group of inherited retinal degenerative disorders caused by mutations in at least 18 different genes. It is the most common cause of inherited childhood blindness, with an incidence of two to three per 100,000 live births worldwide. Symptoms of LCA appear within the first years of life, resulting in significant vision loss and potentially blindness. The most common form of the disease, LCA10, is a monogenic disorder caused by mutations in the CEP290 gene and is the cause of disease in approximately 20-30 percent of all LCA patients.

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 AGN-151587 (EDIT-101). 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 AGN-151587 globally for the treatment of LCA10. Additionally, Editas Medicine exercised its option to co-develop and share equally in the profits and losses from AGN-151587 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/Cpf1 (also known as Cas12a).

About Allergan plc
Allergan plc (NYSE: AGN), headquartered in Dublin, Ireland, is a bold, global pharmaceutical leader. Allergan is focused on developing, manufacturing and commercializing branded pharmaceutical, device, biologic, surgical and regenerative medicine products for patients around the world.

Allergan markets a portfolio of leading brands and best-in-class products primarily focused on four key therapeutic areas including medical aesthetics, eye care, central nervous system and gastroenterology.

Allergan is an industry leader in Open Science, a model of research and development, which defines our approach to identifying and developing game-changing ideas and innovation for better patient care. With this approach, Allergan has built one of the broadest development pipelines in the pharmaceutical industry.

Allergan’s success is powered by our global colleagues’ commitment to being Bold for Life. Together, we build bridges, power ideas, act fast and drive results for our customers and patients around the world by always doing what is right.

With commercial operations in approximately 100 countries, Allergan is committed to working with physicians, healthcare providers and patients to deliver innovative and meaningful treatments that help people around the world live longer, healthier lives every day.

For more information, visit Allergan's website at www.Allergan.com.

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 (also known as 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.

Allergan Forward-Looking Statements

Statements contained in this press release that refer to future events or other non-historical facts are forward-looking statements that reflect Allergan's current perspective on existing trends and information as of the date of this release. Actual results may differ materially from Allergan's current expectations depending upon a number of factors affecting Allergan's business. These factors include, among others, the difficulty of predicting the timing or outcome of FDA approvals or actions, if any; the impact of competitive products and pricing; market acceptance of and continued demand for Allergan's products; the impact of uncertainty around timing of generic entry related to key products, including RESTASIS®, on our financial results; risks associated with divestitures, acquisitions, mergers and joint ventures; risks related to impairments; uncertainty associated with financial projections, projected cost reductions, projected debt reduction, projected synergies, restructurings, increased costs, and adverse tax consequences; difficulties or delays in manufacturing; and other risks and uncertainties detailed in Allergan's periodic public filings with the Securities and Exchange Commission, including but not limited to Allergan's Annual Report on Form 10-K for the year ended December 31, 2018. Except as expressly required by law, Allergan disclaims any intent or obligation to update these forward-looking statements.

Editas Medicine 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 Companies' plans with respect to the planned Phase 1/2 clinical trial for AGN-151587 (EDIT-101), including initiating patient screening by mid-year and dosing patients in the second half of 2019. Editas Medicine 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 Editas Medicine'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 Editas Medicine'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 Editas Medicine's most recent Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, and in other filings that Editas Medicine 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 Editas Medicine expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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Source: Editas Medicine, Inc.