Allergan and Editas Medicine Announce Dosing of First Patient in Landmark Phase 1/2 Clinical Trial of CRISPR Medicine AGN-151587 (EDIT-101) for the Treatment of LCA10

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AGN-151587 (EDIT-101) is the first in vivo CRISPR medicine to be administered to patients

Additional patient enrollment to the BRILLIANCE Clinical Trial is ongoing

DUBLIN, Ireland and CAMBRIDGE, Mass., March 04, 2020 (GLOBE NEWSWIRE) -- Allergan plc (NYSE: AGN), a leading global pharmaceutical company, and Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced the treatment of the first patient in the BRILLIANCE clinical trial of AGN-151587 (EDIT-101) at Oregon Health & Science University (OHSU) Casey Eye Institute, a world-recognized academic eye center.

AGN-151587 (EDIT-101) is an experimental medicine delivered via sub-retinal injection under development for the treatment of Leber congenital amaurosis 10 (LCA10), an inherited form of blindness caused by mutations in the centrosomal protein 290 (CEP290) gene. The BRILLIANCE clinical trial is a Phase 1/2 study to evaluate AGN-151587 for the treatment of patients diagnosed with LCA10 and is the world’s first human study of an in vivo, or inside the body, CRISPR genome editing medicine. The trial will assess the safety, tolerability, and efficacy of AGN-151587 in approximately 18 patients with LCA10.

“This dosing is a truly historic event – for science, for medicine, and most importantly for people living with this eye disease,” said Cynthia Collins, President and CEO, Editas Medicine. “The first patient dosed in the BRILLIANCE clinical trial marks a significant milestone toward delivering on the promise and potential of CRISPR medicines to durably treat devastating diseases such as LCA10. We look forward to sharing future updates from this clinical trial and our ocular program.”

“Currently patients living with LCA10 have no approved treatment options. For years, Allergan has had an unwavering commitment to advancing eye care treatments. With the first patient treated in this historic clinical trial, we mark a significant step in advancing the AGN-151587 clinical program and move closer to our goal of developing a game-changing medicine for LCA10 patients,” said Brent Saunders, Chairman and CEO, Allergan.

“Our first treatment in this clinical trial is an important step toward bringing new and promising treatments to patients with disease-causing gene mutations. OHSU is honored to be involved in this effort to address previously untreatable diseases such as Leber congenital amaurosis 10,” said Mark Pennesi, M.D., Ph.D., Associate Professor of Ophthalmology, Kenneth C. Swan Endowed Professor, Division Chief, Paul H. Casey Ophthalmic Genetics, Casey Eye Institute, Oregon Health & Science University, Principal Investigator and enrolling physician of the first patient treated with AGN-151587.

Eric A. Pierce, M.D., Ph.D., Director of the Inherited Retinal Disorders Service and Director of the Ocular Genomics Institute at Massachusetts Eye and Ear, and the William F. Chalots Professor of Ophthalmology at Harvard Medical School, and a Principal Investigator for the BRILLIANCE clinical trial also commented, “We have a long history at Massachusetts Eye and Ear of helping develop life-changing medicines for our patients, and we are thrilled to be a leader in the development of a CRISPR-based experimental medicine to treat CEP290-associated retinal disease with Allergan and Editas.”

About the BRILLIANCE Phase 1/2 Clinical Trial of AGN-151587 (EDIT-101)

The BRILLIANCE Phase 1/2 clinical trial of AGN-151587 (EDIT-101) for the treatment of Leber congenital amaurosis 10 (LCA10) will assess the safety, tolerability, and efficacy of AGN-151587 in approximately 18 patients with this disorder. Up to five cohorts of patients across three dose levels will be enrolled in this open label, multi-center, clinical trial. Both adult and pediatric patients (3 – 17 years old) with a range of baseline visual acuity assessments are eligible for enrollment. Patients will receive a single administration of AGN-151587 via subretinal injection in one eye. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

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 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. Editas Medicine is also eligible to receive development and commercial milestone payments, 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). 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.

About Allergan plc
Allergan plc (NYSE: AGN), headquartered in Dublin, Ireland, is a global pharmaceutical leader 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. As part of its approach to delivering innovation for better patient care, Allergan has built one of the broadest pharmaceutical and device research and development pipelines in the industry.

With colleagues and commercial operations located 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/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.

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, 2019. 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 Phase 1/2 clinical trial for AGN-151587 (EDIT-101). 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, 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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