Biogen Announces Enrollment Completion of Global Phase 3 Gene Therapy Study for an Inherited Retinal Disorder

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Timrepigene emparvovec is a potential first-in-class AAV2 gene therapy for the treatment of choroideremia, a rare, degenerative, X-linked retinal disorder that leads to blindness

CAMBRIDGE, Mass., Nov. 21, 2019 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced today the enrollment of the last patient in the global Phase 3 STAR clinical study, which is evaluating the investigational gene therapy timrepigene emparvovec (BIIB111/AAV2-REP1) for the treatment of choroideremia (CHM). CHM is a rare, degenerative, X-linked inherited retinal disorder that leads to blindness.

"We are excited to advance innovative investigational treatments for inherited retinal disorders that have significant unmet medical need due to the lack of treatment options," said Alfred Sandrock, Jr., M.D., Ph.D., Executive Vice President, Research and Development, and Chief Medical Officer at Biogen. "Timrepigene emparvovec could be a transformative gene therapy for individuals living with choroideremia who would otherwise face inevitable blindness. Completing enrollment of our Phase 3 study represents a significant milestone in bringing this new potential therapy to patients."

STAR is a randomized, masked, prospective, parallel-controlled group Phase 3 study that enrolled 170 adult males with CHM. The study is evaluating the safety and efficacy of a single subretinal injection of timrepigene emparvovec. The primary endpoint is the proportion of patients with an improvement of at least 15 letters from baseline in best corrected visual acuity (BCVA) at 12 months post treatment as measured by the Early Treatment Diabetic Retinopathy Study (ETDRS) visual acuity protocol. The STAR study was initiated based on proof-of-concept data from Phase 1/2 studies, which indicated that at month 24, over 90 percent of patients treated with timrepigene emparvovec via targeted subretinal injection maintained visual acuity instead of experiencing the natural decline in BCVA expected in this degenerative disease. In a subset of treated patients with moderate to severe visual acuity loss, 21 percent experienced a gain in visual acuity of at least 15 ETDRS letters from baseline as compared to one percent of untreated patients in a natural history study.

CHM primarily affects males and is caused by a loss of function in the CHM gene which encodes the Rab escort protein-1 (REP-1). The REP-1 protein plays a role in intracellular protein trafficking, and the loss of function in the CHM gene leads to abnormal intracellular protein trafficking and impaired elimination of waste products from the retinal pigment epithelium and photoreceptors. Initially, patients with CHM experience poor night vision and over time, progressive visual loss ultimately leads to blindness.

Biogen added timrepigene emparvovec to its portfolio in June 2019 as part of its acquisition of Nightstar Therapeutics.

For more information about the Phase 3 STAR study, visit www.clinicaltrials.gov (NCT03496012).

About timrepigene emparvovec (BIIB111/AAV2-REP1)
Timrepigene emparvovec is an AAV2 vector administered by subretinal injection, which aims to provide a functioning CHM gene and expression of the REP-1 protein to restore membrane trafficking and thereby slow, stop or potentially reverse decline in vision. Data from the Phase 1/2 studies demonstrated a slower rate of decline in visual acuity in patients treated with timrepigene emparvovec compared to untreated patients in the natural history study. In addition, some patients treated with timrepigene emparvovec showed improvements in visual acuity. The studies also demonstrated that timrepigene emparvovec was generally well tolerated with an acceptable safety profile.

Timrepigene emparvovec has received regenerative medicine advanced therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA), which includes all of the benefits of the fast track and breakthrough therapy designation programs and orphan drug designations in the U.S., Europe and Japan. The safety and efficacy of a single subretinal injection of timrepigene emparvovec is currently being evaluated in the ongoing Phase 3 STAR study.

About Biogen
At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics, and is focused on advancing research programs in multiple sclerosis and neuroimmunology, neuromuscular disorders, movement disorders, Alzheimer's disease and dementia, ophthalmology, immunology, neurocognitive disorders, acute neurology, and pain.

We routinely post information that may be important to investors on our website at www.biogen.com. To learn more, please visit www.biogen.com and follow us on social media – Twitter, LinkedIn, Facebook, YouTube.

Biogen Safe Harbor Statement
This news release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, relating to the potential, benefits, safety and efficacy of timrepigene emparvovec; the potential clinical effects of timrepigene emparvovec; results from the Phase 1/2 studies of timrepigene emparvovec; the clinical development program for timrepigene emparvovec; the treatment of CHM; the potential of our commercial business and pipeline programs, including timrepigene emparvovec; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "will," "would" and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements, or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including

without limitation, uncertainty of success in the development and potential commercialization of timrepigene emparvovec; unexpected concerns may arise from additional data, analysis or results obtained during the STAR study; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including timrepigene emparvovec; the occurrence of adverse safety events; the risks of other unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; and product liability claims. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this news release. We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments or otherwise.

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