Biogen Announces Topline Results from Phase 3 Gene Therapy Study in Choroideremia

June 14, 2021

- *The Phase 3 STAR study did not meet primary or key secondary endpoints*
- *Choroideremia is a rare inherited retinal disease that results in progressive vision loss, ultimately leading to blindness*

CAMBRIDGE, Mass., June 14, 2021 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced topline results from the Phase 3 STAR study of timrepigene emparvovec (BIIB111/AAV2-REP1), an investigational gene therapy for the potential treatment of choroideremia. The STAR study did not meet its primary endpoint of proportion of participants with a ≥15 letter improvement from baseline in best corrected visual acuity (BCVA) at Month 12, in the interventional group in comparison to the non-interventional control group, as measured by the Early Treatment of Diabetic Retinopathy Study (ETDRS) chart. In addition, the study did not demonstrate efficacy on key secondary endpoints. Safety results from the Phase 3 STAR study were consistent with previous studies.

"We extend our deepest gratitude to all those who contributed to the STAR study, including the participants, investigators, site staff and the broader choroideremia community," said Katherine Dawson, M.D., head of the Therapeutics Development Unit at Biogen. "While we are disappointed by the results of the STAR study, we are hopeful that the clinical insights gleaned from this study may help to shape therapeutic innovation for inherited retinal diseases including choroideremia, so that in the future there may be treatment options for the community affected by these debilitating disorders."

Biogen will evaluate the complete data set before confirming next steps for the timrepigene emparvovec clinical development program. Detailed results of this study will be made available at a future scientific forum.

About timrepigene emparvovec (BIIB111/AAV2-REP1)
Timrepigene emparvovec is an investigational recombinant AAV2 vector designed to deliver a functional version of the human choroideremia gene into the retinal pigment epithelium and photoreceptor cells that aims to address the underlying genetic cause of choroideremia.

About the STAR Phase 3 Study (NCT03496012)
STAR was a Phase 3, multicenter, randomized, three-arm, parallel-controlled group study that enrolled 169 adult males with a genetically confirmed diagnosis of choroideremia. The study evaluated the safety and efficacy of a single subretinal injection of investigational timrepigene emparvovec. The primary endpoint was 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) chart. While the Phase 3 STAR study did not meet its primary endpoint, more information about the study is available here: www.clinicaltrials.gov.

About Choroideremia
Choroideremia is a rare, inherited retinal disease resulting in progressive vision loss, ultimately leading to blindness. Choroideremia is an X-linked recessive chorioretinal disease that is caused by loss of function mutations in the choroideremia gene resulting in decreased Rab escort protein-1 (REP-1) expression which leads to degeneration of the retinal pigment epithelium, photoreceptors and choroid. Initially, patients with choroideremia experience poor night vision and over time, slow, progressive visual loss ultimately leads to blindness. Visual impairments due to choroideremia are associated with emotional, functional and economic burden.

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, Alzheimer's disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, neuropsychiatry, immunology, acute neurology and neuropathic pain.

We routinely post information that may be important to investors on our website at www.biogen.com. 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 clinical effects and safety of timrepigene emparvovec; results from the Phase 3 study of timrepigene emparvovec; the clinical development program for timrepigene emparvovec; the potential treatment of inherited retinal diseases including choroideremia; 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 of timrepigene emparvovec; unexpected concerns may arise from additional data, analysis or results obtained during clinical trials, including the STAR study; 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; product liability claims; and the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations and financial condition. 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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