Biogen Provides Update on FDA Advisory Committee Meeting on Tofersen for SOD1-ALS

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SOD1-ALS is a rare genetic form of the disease affecting approximately 330 people in the United States

CAMBRIDGE, Mass., March 22, 2023 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced today the outcome of the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee meeting on tofersen, an investigational product for the treatment of superoxide dismutase 1 (SOD1) amyotrophic lateral sclerosis (ALS).

On the question, "Is the available evidence sufficient to conclude that a reduction in plasma neurofilament light chain (NfL) concentration in tofersen-treated patients is reasonably likely to predict clinical benefit of tofersen for treatment of patients with SOD1-ALS?" the Committee voted unanimously yes (9 yes to 0 no), for consideration of a potential accelerated approval.

On the second question, "Does the clinical data from the placebo-controlled study and available long-term extension study results, with additional supporting results from the effects on relevant biomarkers (i.e., changes in plasma NfL concentration and/or reductions in SOD1), provide substantial evidence of the effectiveness of tofersen in the treatment of patients with SOD1-ALS?" the Committee voted 3 (yes), 5 (no) and 1 (abstain), for consideration of a potential traditional approval.

Additionally, the committee discussed both of these topics and reached consensus that the benefit-risk profile was favorable based on the review of the totality of data for tofersen in people with SOD1-ALS.

"After hearing the moving experiences of the ALS community and reviewing the totality of data, the Committee voted that reductions of neurofilament are reasonably likely to predict clinical benefit of tofersen. If approved, tofersen would potentially represent a major advance for people living with SOD1-ALS," said Priya Singhal, M.D., M.P.H, Executive Vice President and Head of Development and interim Head of Research and Global Safety and Regulatory Sciences at Biogen. "We thank the FDA for convening this important discussion. Most importantly, we are grateful to all the people with SOD1-ALS who participated in our tofersen studies, and their caregivers, families, study investigators and the entire community, without whom this scientific progress could not have been made."

FDA Advisory Committees provide non-binding recommendations for consideration by the FDA. The New Drug Application for tofersen for the treatment of SOD1-ALS was submitted to the FDA for consideration under accelerated approval. The FDA is continuing its review of tofersen with a Prescription Drug User Fee Act action date of April 25, 2023.

About Tofersen
Tofersen is an antisense oligonucleotide (ASO) being evaluated as a treatment for SOD1-ALS. In people with this form of the disease, mutations in their SOD1 gene cause their bodies to create a toxic form of SOD1 protein. This toxic protein causes motor neurons to degenerate, resulting in progressive muscle weakness. Tofersen is designed to bind to SOD1 mRNA and reduce SOD1 protein production.

In addition to the ongoing open label extension of the Phase 3 VALOR study, tofersen is being studied in the Phase 3 ATLAS study designed to evaluate whether tofersen can delay clinical onset when initiated in presymptomatic individuals with a SOD1 genetic mutation and biomarker evidence of disease activity. Biogen licensed tofersen from Ionis Pharmaceuticals, Inc. under a collaborative development and license agreement.

About Amyotrophic Lateral Sclerosis and SOD1-ALS
Amyotrophic lateral sclerosis (ALS) is an ultra-rare, progressive and fatal neurodegenerative disease that results in the loss of motor neurons in the brain and the spinal cord that are responsible for controlling voluntary muscle movement. People with ALS experience muscle weakness and atrophy, causing them to lose independence as they steadily lose the ability to move, speak, eat, and eventually breathe. Average life expectancy for people with ALS is three to five years from time of symptom onset.[1]

Multiple genes have been implicated in ALS. Genetic testing helps determine if a person's ALS is associated with a genetic mutation, even in individuals without a family history of the disease. Currently, there are no genetically targeted treatment options for ALS. SOD1-ALS is diagnosed in approximately 2 percent of all ALS cases, impacting about 330 people in the United States.[2] While there are medications approved for broad ALS, no available treatments target a genetic mutation associated with ALS. Approximately 5-10 percent of people with ALS are thought to have a genetic form of the disease;[1] however, they may not have a known family history of the disease.

Biogen's Continuous Commitment to ALS
For over a decade, Biogen has been committed to advancing ALS research to provide a deeper understanding of all forms of the disease. The company has continued to invest in and pioneer research despite making the difficult decision to discontinue a late-stage ALS asset in 2013. Biogen has applied important learnings to its portfolio of assets for genetic and other forms of ALS, with the goal of increasing the probability of bringing a potential therapy to patients in need. These applied learnings include evaluating genetically validated targets in defined patient populations, pursuing the most appropriate modality for each target and employing sensitive clinical endpoints. Today, the company has a pipeline of investigational drugs being evaluated in ALS, including tofersen and BIIB105.

About Biogen
Founded in 1978, Biogen is a leading global biotechnology company that has pioneered multiple breakthrough innovations including a broad portfolio of medicines to treat multiple sclerosis, the first approved treatment for spinal muscular atrophy, and two co-developed treatments to address a defining pathology of Alzheimer's disease. Biogen is advancing a pipeline of potential novel therapies across neurology, neuropsychiatry, specialized immunology and rare diseases and remains acutely focused on its purpose of serving humanity through science while advancing a healthier, more sustainable and equitable world.

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

This news release contains forward-looking statements, including statements about results from the Phase 3 VALOR study of tofersen or its OLE; the potential clinical effects of tofersen; the potential benefits, safety and efficacy of tofersen; the clinical development program for tofersen; the potential approval of tofersen; the identification and treatment of ALS; our research and development program for the treatment of ALS; the potential of our commercial business and pipeline programs, including tofersen; 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 our forward-looking statements.

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 tofersen; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; 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 tofersen; the occurrence of adverse safety events; the risks of 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 speak only as of the date of this news release.

We do not undertake any obligation to publicly update any forward-looking statements.

References:

1. National Institute of Neurological Disorders and Stroke. Amyotrophic Lateral Sclerosis (ALS) Fact Sheet. Available at: https://www.ninds.nih.gov/amyotrophic-lateral-sclerosis-als-fact-sheet. Accessed: March 2023.
2. Brown CA, Lally C, Kupelian V, Flanders WD. Estimated Prevalence and Incidence of Amyotrophic Lateral Sclerosis and SOD1 and C9orf72 Genetic Variants. Neuroepidemiology. 2021;55(5):342-353. doi: 10.1159/000516752. Epub 2021 Jul 9.

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