European Medicines Agency Accepts Tofersen Marketing Authorization Application to Treat Rare, Genetic Form of ALS

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- SOD1-ALS is a rare, genetic form of ALS,[1] comprising approximately 2% of people with ALS[2]
- If approved, tofersen would be the first treatment to target a genetic cause of ALS

CAMBRIDGE, Mass., Dec. 05, 2022 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced the European Medicines Agency (EMA) has accepted the Marketing Authorization Application (MAA) for review of tofersen, an investigational drug for the treatment of superoxide dismutase 1 (SOD1) amyotrophic lateral sclerosis (ALS). SOD1-ALS is a progressive and uniformly fatal disease that affects less than 1,000 people in Europe.[2] There is currently no treatment targeted for SOD1-ALS.[3]

“Through our clinical development program, we have seen that tofersen has the potential to slow the progression of this relentless and ultimately fatal disease,” said Priya Singhal, M.D., M.P.H., Head of Global Safety and Regulatory Sciences and Interim Head of R&D at Biogen. “Regulatory submissions in the U.S. and now EU represent an important step in our efforts to bring the first genetically-targeted treatment for SOD1-ALS to the ALS community as quickly as possible.”

The MAA includes results from the Phase 3 VALOR study, its open label extension (OLE) study, a Phase 1 study in healthy volunteers and a Phase 1/2 study evaluating ascending dose levels. Also included are the most current 12-month integrated results from VALOR and the OLE study that were recently published in *The New England Journal of Medicine*.

“The effects we have seen with tofersen diverge from the natural history of the disease and bring hope for the treatment of SOD1-ALS,” said Philip Van Damme, M.D., Ph.D., professor of neurology and director of the Neuromuscular Reference Center at the University Hospital Leuven in Belgium. “Today’s announcement is an important milestone for the ALS community in Europe where there is a tremendous need for additional treatment options.”

Tofersen is also under review with the U.S. Food and Drug Administration with Priority Review and has a Prescription Drug User Fee Act action date of April 25, 2023.

Biogen will maintain its early access program for tofersen, which is now available in 34 countries. Biogen continues to actively engage with other regulators around the world and will provide updates when appropriate.

About Tofersen
Tofersen is an antisense drug being evaluated as a treatment of SOD1-ALS. Tofersen is designed to bind to SOD1 mRNA, inducing its degradation by RNase-H to reduce SOD1 protein production. In addition to the ongoing open label extension of VALOR, 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 a 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.[3]

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. Mutations in the SOD1 gene are responsible for approximately 2 percent of the estimated 168,000 people who have ALS globally (SOD1-ALS).[2] Life expectancy in SOD1-ALS varies widely with some patients surviving less than a year.[4]

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
As pioneers in neuroscience, Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological 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, Sir Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today, Biogen has a leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and developed the first and only approved treatment to address a defining pathology of Alzheimer’s disease. Biogen is also commercializing biosimilars and focusing on advancing one of the industry’s most diversified pipelines in neuroscience that will transform the standard of care for patients in several areas of high unmet need.

In 2020, Biogen launched a bold 20-year, $250 million initiative to address the deeply interrelated issues of climate, health, and equity. Healthy Climate, Healthy Lives™ aims to eliminate fossil fuels across the company’s operations, build collaborations with renowned institutions to advance the science to improve human health outcomes, and support underserved communities.

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 made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, 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 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 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 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.

References:

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