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

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

Biogen 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.

SOURCE: **WEBSITE BIOGEN**

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