October 18, 2021

Dear Chris,

In response to your request for information, we are writing to let you know that, at the American Neurological Association 2021 Annual Meeting on Sunday 17th October, Biogen shared topline results from its pivotal Phase 3 VALOR (233AS101) study and its Open label extension (OLE) study (233AS102). The aim of the VALOR study was to evaluate the safety, efficacy, and tolerability of tofersen (BIIB067) in people with SOD1-ALS, a rare form of amyotrophic lateral sclerosis (ALS) caused by mutations in the SOD1 gene. The study also looked at biomarkers of disease activity and how the body responds to the drug at a biochemical level.

Data indicate that the VALOR study did not meet its primary endpoint of change from baseline to week 28 in ALSFRS. This means that treatment with tofersen did not result in improvements in the functional status of patients with ALS as measured by the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R). However, there were some trends when looking at other measures of disease activity, showing improvements in motor function, respiratory function, and quality of life.

Most adverse events in both VALOR and the open label extension study were mild to moderate in severity. The most common adverse events were pain related to lumbar puncture procedure, headache, pain in limbs, falls, and back pain.

Biogen is engaging with regulators, the medical community, patient advocacy groups and other key stakeholders around the world to determine potential next steps.

In light of the critical unmet need, Biogen will broaden its ongoing early access programme (EAP) to all people with SOD1-ALS who meet the eligibility criteria, in countries where such programmes are permitted by local regulations and future access may be secured. This includes the UK, and Biogen continues to have a dialogue with licensing and regulatory authorities in this country. If a path forward for tofersen is not established, or if another controlled trial is required by regulators, Biogen may revise or discontinue the EAP.

We extend our deepest and most sincere gratitude to each of the study participants and their families, clinical site staff, physicians and broader ALS advocacy community who contributed to the VALOR study.

Yours sincerely,

Mihaela Vlaicu, MD
Sr. Director, Head of Medical, Biogen UK and Ireland

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