23 February 2024

Dear European Organization for Professionals and Patients with ALS,

Thank you for your request to receive updates about QALSODY® (tofersen). We are writing with the news that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency adopted a positive opinion recommending a marketing authorization under exceptional circumstances for tofersen for the treatment of adults with amyotrophic lateral sclerosis (ALS), associated with a mutation in the superoxide dismutase 1 (*SOD1*) gene.

A marketing authorization under exceptional circumstances is recommended when the benefit/risk assessment is determined to be positive but due to the rarity of the disease, it is unlikely that comprehensive data can be obtained under normal conditions of use.

Summary of the CHMP Opinion

The benefits of tofersen are a reduction in the levels of *SOD1* in the cerebrospinal fluid, a reduction in the levels of plasma neurofilament light chain (a marker of neuronal damage), and a numerically favourable effect on the ALS Functional Ratings Scale-Revised (ALSFRS-R), which is used to score the physical abilities of patients. The most common side effects with tofersen are pain, fatigue, pyrexia, arthralgia, myalgia and increased levels of white blood cells and proteins in the cerebrospinal fluid.

The full indication is:
Tofersen is indicated for the treatment of adults with amyotrophic lateral sclerosis (ALS), associated with a mutation in the superoxide dismutase 1 (*SOD1*) gene.

Next Steps

The CHMP's recommendation for tofersen will now be reviewed by the European Commission (EC) for marketing authorization in the European Union with a final decision expected in the second quarter of 2024. A positive EC decision would mean that tofersen is approved as the first treatment for *SOD1*-ALS in the European Union.

As we work to bring tofersen to people living with *SOD1*-ALS, we thank the ALS community for your efforts to support those living with the condition.

Regards,
Cindy

Cindy McGee Manieri
Head of Corporate Affairs Rare Disease, International