22 February 2024
EMA/CHMP/50520/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Qalsody

tofersen

On 22 February 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Qalsody[3], intended for the treatment of a type of amyotrophic lateral sclerosis caused by a defective superoxide dismutase 1 (SOD1) protein.

The Applicant for this medicinal product is Biogen Netherlands B.V.

Qalsody will be available as a 100 mg solution for injection and will be given intrathecally by lumbar puncture. The active substance of Qalsody is tofersen, an other nervous system drug (ATC code: N07XX22). Tofersen is an antisense oligonucleotide that binds to the mRNA of the superoxide dismutase 1 (*SOD1*) gene, leading to its breakdown and a reduction in the amount of SOD1 protein produced.

The benefits of Qalsody 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 ALSFRS-R scale which is used to score the physical abilities of patients. The most common side effects with Qalsody are pain, fatigue, pyrexia, arthralgia, myalgia and increased levels of white blood cells and proteins in the cerebrospinal fluid.

The full indication is:

> Qalsody is indicated for the treatment of adults with amyotrophic lateral sclerosis (ALS), associated with a mutation in the superoxide dismutase 1 (SOD1) gene.

Qalsody should be prescribed by physicians experienced in the treatment of ALS.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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available in all official European Union languages after the marketing authorisation has been granted by the European Commission.