swissmedic

Summary report on authorisation dated 10 April 2026

Qalsody® (active substance: tofersen)

Temporary authorisation in Switzerland: 19 December 2025

Solution for injection for the treatment of amyotrophic lateral sclerosis (ALS) associated with a mutation in the superoxide dismutase 1 gene

About the medicinal product

Qalsody contains the active substance tofersen and is used to treat adult patients with amyotrophic lateral sclerosis (ALS) caused by a mutated gene called superoxide dismutase 1 (SOD1).

The mutated gene leads to a build-up of the protein SOD1, which is toxic to nerve cells. This results in a loss of nerve cells in the brain and spinal cord, with consequent weakness of the muscles, including those responsible for breathing and swallowing.

Since this is a rare and life-threatening disease that affects around 2 % of ALS patients, the medicinal product Qalsody has been authorised as an orphan drug. "Orphan drug" is a designation given to medicinal products for rare diseases.

Mode of action

The active substance of the medicinal product Qalsody consists of laboratory-produced genetic material (called antisense oligonucleotide) that binds to the SOD1 genetic material in the patients' nerve cells, thereby blocking the formation of mutated SOD1 protein.

This mode of action reduces the amount of accumulated SOD1 protein, and thus helps prevent the loss of nerve cells and slow the loss of muscle strength.

Administration

Qalsody is available only on prescription as a solution for injection.

A 15 ml vial contains 6.7 mg/ml of the active substance tofersen.

The medicinal product Qalsody is administered to patients by experienced healthcare

professionals via a lumbar puncture[1] performed three times at 14-day intervals, followed by treatment every 28 days.

Efficacy

The efficacy of Qalsody was investigated in a 28-week study involving a total of 108 patients with ALS and a confirmed SOD1 mutation.

Over 24 weeks, the patients taking part in the study received either the medicinal product Qalsody, containing the active substance tofersen, or a dummy drug (placebo).

In a follow-up study, the patients continued their treatment with Qalsody.

Although key measured indicators of treatment success in the first study did not show a statistically significant improvement, the overall results of the studies showed that patients benefit from treatment with Qalsody and that the further progression of the disease can be slowed.

Precautions, undesirable effects, & risks

The medicinal product Qalsody must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects are back pain, pain in the arms or legs, increase in protein and/or the white blood cell count in the fluid that surrounds the brain and spinal cord, fatigue, muscle and joint pain, and fever.

Serious side effects can occur during the administration of Qalsody. All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

At the time of authorisation of Qalsody, only limited treatment options were available for patients suffering from ALS with a mutation in the SOD1 gene. Consequently, the medical need for effective medicines is high.

Taking all the risks and precautions into account, and based on the available data, the benefits of Qalsody outweigh the risks.

The medicinal product Qalsody has been authorised temporarily in Switzerland (in accordance with Art. 9a TPA) since not all clinical trials were available or had been concluded at the time of authorisation.

The temporary authorisation is contingent on the timely submission of the data requested by Swissmedic. Once these authorisation conditions have been met, the temporary authorisation can be converted into an authorisation without special conditions in the event of a positive benefit-risk assessment of the results.

[1] Lumbar puncture: Injection into the lumbar segment of the spinal canal

Further information on the medicinal product

At the time of publication of the Summary report on authorisation for Qalsody, the Information for healthcare professionals and the Patient information were not yet available. As soon as the medicinal product becomes available in Switzerland, the package leaflet and the Information for healthcare professionals will be made available on the following website: www.swissmedicinfo.ch Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.