swissmedic

Summary report on authorisation dated 15 November 2024

Libmeldy® (active substance: atidarsagene autotemcel)

Authorisation in Switzerland: 7 December 2023

Dispersion for infusion for the treatment of children with metachromatic leukodystrophy (MLD).

About the medicinal product

Libmeldy contains the active substance atidarsagene autotemcel.

The medicinal product Libmeldy is used for the treatment of children with metachromatic leukodystrophy (MLD).

Libmeldy is used in:

- children with late infantile or early juvenile MLD who have not yet developed symptoms
- children with early juvenile MLD who have initial symptoms but still have the ability to walk independently and whose cognitive function has not yet deteriorated.

MLD is a rare genetic disease. It is characterised by a change (mutation) in the gene required to produce the enzyme arylsulfatase A (ARSA). ARSA is needed to break down certain substances called sulfatides. This genetic change leads to a reduction in the enzyme activity of ARSA. As a result, sulfatides accumulate, which can damage the nervous system and other organs.

Children with MLD can have difficulties walking and the genetic defect can cause a gradual decline in cognitive function, which may ultimately lead to death.

Since MLD is a rare and life-threatening disease, Libmeldy has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Libmeldy has been authorised by Swissmedic under Article 13 of the Therapeutic Products Act (TPA). This means that the medicinal product is already authorised in another country with comparable medicinal product control. In this case, Swissmedic takes into consideration the results of checks carried out by foreign regulatory agencies, provided certain requirements are fulfilled. These involve checks on the quality, efficacy, and safety of the medicinal product, and the extent to which the results can be accepted for Switzerland. The consideration of the results of foreign authorisation procedures is intended to help ensure that medicines that are already authorised abroad can be made available to patients in Switzerland as quickly as possible.

In deciding whether to authorise Libmeldy in Switzerland, Swissmedic accepted the assessment and approval decision of the European Medicines Agency (EMA; reference number EMA/584450/2020) and has only conducted a limited scientific review.

Accordingly, in the SwissPAR (Swiss Public Assessment Report) and the resulting Summary report on authorisation, Swissmedic refers to the Assessment Report issued by the reference authority:
www.ema.europa.eu

Further information on the medicinal product

At the time of publication of the summary report on authorization, the Information for healthcare professionals and the Patient information (package leaflet) were not yet available. As soon as the medicine is available in Switzerland, the Information for healthcare professionals and the Patient information will be made available online at the following address:

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.