swissmedic

Summary report on authorisation dated 25 October 2024

Pombiliti® (active substance: cipaglucosidase alfa)

Authorisation in Switzerland: 4 July 2024

Powder for concentrate for solution for infusion for the treatment of adults with late-onset Pompe disease

About the medicinal product

Pombiliti contains the active substance cipaglucosidase alfa and is used for long-term enzyme replacement therapy in adults with late-onset Pompe disease. This disease is caused by a genetic lack of the enzyme "acid alpha glucosidase" in muscle cells. This enzyme is essential for breaking down glycogen, which is a storage form of glucose in the body. If glycogen is not broken down, it can accumulate in various muscle cells such as those of the heart or diaphragm. As a result, the patient experiences heart problems, breathing difficulties and muscle weakness.

Pombiliti is given in combination with another medicinal product (Miglustat). Pombiliti enters the affected muscle cells, where it supports the breakdown of glycogen.

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

Pombiliti has been authorised by Swissmedic under Article 13 of the Therapeutic Products Act (TPA). This means that the medicinal product is already authorised in at least one other 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 Pombiliti in Switzerland, Swissmedic accepted the assessment and approval decision of the European Medicines Agency (EMA; reference number EMA/950090/2022) 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

Information for healthcare professionals: Information for healthcare professionals Pombiliti®

Information for patients (package leaflet): Information for patients Pombiliti®

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.