

Public Summary SwissPAR dated 29 April 2022

Nityr[®] (active substance: nitisinone)

First authorisation in Switzerland: 24 January 2022

Medicinal product (tablet) for the treatment of hereditary tyrosinemia type 1 (HT-1) in children, adolescents and adults.

Information on authorisation

The medicinal product Nityr contains the active substance nitisinone. It is available as a tablet in three dosages.

Nityr has been authorised in Switzerland for the treatment of hereditary tyrosinemia type 1 (HT-1) in children, adolescents and adults. Patients must also follow a restricted diet.

Patients with HT-1 lack an enzyme¹ to effectively break down the amino acid² tyrosine. As a result, it is converted into harmful substances. Nityr blocks the enzymes that convert tyrosine into harmful substances. The additional dietary restrictions also prevent excessive concentrations of tyrosine and other amino acids that can be converted into tyrosine in the body.

Nityr was approved under a simplified authorisation procedure according to Art. 14 para. 1 let. a^{bis} of the Therapeutic Products Act (TPA). The TPA enables certain categories of medicines to be authorised according to a simplified procedure, provided this is

compatible with the quality, safety and efficacy requirements and there is no conflict with Swiss interests or international obligations.

The authorisation of Nityr is based on the medicinal product Orfadin, which contains the same active substance and has been authorised for a comparable indication, dosage and use in Germany for more than 10 years.

Swissmedic assessed the quality data on the active substance and finished product but did not conduct its own comprehensive scientific review for other aspects. Efficacy and safety were only reviewed in summarised form.

The requirements for issuing a complete SwissPAR (Swiss Public Assessment Report) and the resulting Public Summary SwissPAR have therefore not been met. Swissmedic refers to the authorisation of the foreign comparator medicinal product:

<https://www.ema.europa.eu>

¹ Enzymes: enzymes are proteins that act as biocatalysts, controlling and accelerating biochemical reactions in the body.

² Amino acids: amino acids are building blocks which the body can use to manufacture proteins.

Further information on simplified authorisation according to Art. 14 TPA can be found in the Federal Act on Medicinal Products and Medical Devices (Therapeutic Products Act, TPA).

Since this is a rare and life-threatening disease, the medicine has been authorised as an orphan drug. The term "orphan drug" refers to important medicines for rare diseases.

Further information on the medicinal product

At the time of publication of the Public Summary SwissPAR for Nityr, the Information for healthcare professionals and the Patient information (package leaflet) were not yet available. As soon as the medicine becomes

available in Switzerland, the Information for healthcare professionals and the Patient information 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 Public Summary SwissPAR.

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.