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Public summary of opinion on orphan designation

Trehalose for the treatment of mucopolysaccharidosis type III

On 21 August 2020, orphan designation EU/3/20/2323 was granted by the European Commission to FGK Representative Service GmbH, Germany, for trehalose for the treatment of mucopolysaccharidosis type III.

What is mucopolysaccharidosis III?

Mucopolysaccharidosis type III (also known as Sanfilippo syndrome) is an inherited disease caused by the deficiency of one of four enzymes that break down substances in the body called glycosaminoglycans (GAGs). If one of these enzymes is not present or does not work well, GAGs cannot be broken down and they build up in the cells, particularly in the brain, and damage them. This causes a wide range of symptoms, including behavioural problems, learning disabilities and eventually patients' ability to walk. The disease is usually diagnosed in children between two and six years of age.

Mucopolysaccharidosis type III is a seriously debilitating and life-threatening disease that progresses to serious mental disability. The disease usually leads to death during adolescence or early adulthood.

What is the estimated number of patients affected by the condition?

At the time of designation, mucopolysaccharidosis type III affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for treating mucopolysaccharidosis type III. Patients received supportive treatment to relieve the symptoms of the disease.

*For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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How is this medicine expected to work?

The way trehalose works in patients with mucopolysaccharidosis type III is not fully understood. It is expected that it reduces storage of GAGs, thereby relieving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of trehalose have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with trehalose in patients with mucopolysaccharidosis III were ongoing.

At the time of submission, trehalose was not authorised anywhere in the EU for mucopolysaccharidosis III. Orphan designation of trehalose had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 16 July 2020, recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Trehalose	Treatment of mucopolysaccharidosis type III (Sanfilippo syndrome)
Bulgarian	Трехалоза	Лечение на мукополизахаридоза тип III (синдром на Санфилипо)
Croatian	Trehaloza	Liječenje mukopolisaharidoze tip III (sindrom Sanfilippo)
Czech	Trehalosa	Léčba mukopolysacharidozy typu III (syndrom Sanfilippo)
Danish	Trehalose	Behandling af mucopolysaccharidose type III (Sanfilippo syndrom)
Dutch	Trehalose	Behandeling van mucopolysacharidose type III (Sanfilippo syndroom)
Estonian	Trehaloos	III-tüüpi mukopolüsahharidoosi (Sanfilippo sündroomi) ravi
Finnish	Trehaloosi	Tyypin III (Sanfilippo) mukopolysakkaridoosin hoito
French	Tréhalose	Traitement de la mucopolysaccharidose de type III (maladie de Sanfilippo)
German	Trehalose	Behandlung der Mukopolysaccharidose Typ III (Sanfilippo-Syndrom)
Greek	Τρεχαλόζη	Θεραπεία βλεννοπολυσακχαρίδωσης, τύπου III (σύνδρομο Sanfilippo)
Hungarian	Trehalóz	III típusú mucopolisacharidosis (Sanfilippo szindróma) kezelése
Italian	Trealosio	Trattamento della mucopolisaccaridosi di tipo III (sindrome di Sanfilippo)
Latvian	Trehaloze	III tipa mukopolisaharidozes (Sanfilipo sindroms) ārstēšana
Lithuanian	Trehalozė	Mukopolisacharidozės, III tipo gydymas (Sanfilippo sindromas)
Maltese	Trealożju	Kura tal-mukopolisakkaridożi tat-tip III (sindrome ta' Sanfilippo)
Polish	Trehaloza	Leczenie mukopolisacharydozy, typ III (zespół Sanfilippo)
Portuguese	Trealosa	Tratamento da mucopolissacaridose, tipo III (síndrome de Sanfilippo)
Romanian	Trehaloză	Tratamentul mucopolizaharidozei de tip III (sindromul Sanfilippo)
Slovak	Trehalózy	Liečba mukopolysacharidózy typu III (Sanfilippov syndróm)
Slovenian	Trehaloza	Zdravljenje mukopolisaharidoze vrste III (sindroma Sanfilippo)
Spanish	Trehalosa	Tratamiento de la mucopolisacaridosis tipo III (síndrome de Sanfilippo)
Swedish	Trehalos	Behandling av mukopolysackaridos typ III (Sanfilippos syndrom)
Norwegian	Trehalose	Behandling av mukopolysakkaridose, type IIIB (Sanfilippos syndrom type B)
Icelandic	Trehalósi	Meðferð við slímsykrukvilla gerð IIIB (Sanfilippo B heilkenni)

[1] At the time of designation