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EMA/COMP/345422/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Trehalose for the treatment of spinocerebellar ataxia

On 19 June 2015, orphan designation (EU/3/15/1502) was granted by the European Commission to Dr Ulrich Granzer, Germany, for trehalose for the treatment of spinocerebellar ataxia.

What is spinocerebellar ataxia?

Spinocerebellar ataxia is a condition characterised by progressive problems with movement, coordination and balance (ataxia). There are different types of spinocerebellar ataxia, and depending on the type, people may develop different signs and symptoms, such as speech and swallowing difficulties, muscle stiffness, weakness in the muscles that control eye movement and cognitive (mental) impairment.

Spinocerebellar ataxia is a long-term debilitating condition involving progressive slowing of gait, often associated with poor coordination of speech, hands and eye movement.

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

At the time of designation, spinocerebellar ataxia 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 the treatment of spinocerebellar ataxia. Patients were mainly given supportive treatment aimed at easing the symptoms of the disease.

*Disclaimer: 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

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How is this medicine expected to work?

This medicine is made up of trehalose, a type of sugar which can stabilise proteins and stop them from sticking together. Patients with spinocerebellar ataxia produce abnormal proteins which can stick together and form deposits that cause death of nerve cells. In spinocerebellar ataxia, trehalose is expected to help prevent abnormal proteins from forming deposits, thereby reducing the damage to cells and 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 spinocerebellar ataxia had been started.

At the time of submission, trehalose was not authorised anywhere in the EU for spinocerebellar ataxia. Orphan designation of the medicine had been granted in the United States for spinocerebellar ataxia type 3.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 May 2015 recommending the granting of this designation.

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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

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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 spinocerebellar ataxia
Bulgarian	Трехалоза	Лечение на спиноцеребеларна атаксия
Croatian	Trehaloza	Liječenje spinocerebelarne ataksije
Czech	Trehalosa	Léčba spinocerebelárního ataxie
Danish	Trehalose	Behandling af spinocerebellar ataksi
Dutch	Trehalose	Behandeling van spinocerebellaire ataxie
Estonian	Trehaloos	Spinotserebellaarataksia ravi
Finnish	Trehaloosi	Spinoserebellaarisen ataksian hoito
French	Tréhalose	Traitement de l'ataxie spinocérébelleuse
German	Trehalose	Behandlung der spinozerebellären Ataxie
Greek	Τρεαλόζη	Θεραπεία της νωτιαιοπαρεγκεφαλιδικής αταξίας
Hungarian	Trehalózok	Spinocerebelláris ataxia kezelése
Italian	Trealosio	Trattamento dell'atassia spinocerebellare
Latvian	Trehaloze	Spinocerebellārās ataksijas ārstēšana
Lithuanian	Trehalozė	Spinocerebeliarinės ataksijos gydymas
Maltese	Trehalose	Kura tal-atassja spinoċerebellari
Polish	Trehaloza	Leczenie ataksji rdzeniowo-móżdżkowej
Portuguese	Trealosa	Tratamento de ataxia espinocerebelar
Romanian	Trehaloză	Tratamentul ataxiei spinocerebelare
Slovak	Trehalóza	Liečba spinocerebelárnej ataxie
Slovenian	Trehaloza	Zdravljenje spinocerebelarne ataksije
Spanish	Trehalosa	Tratamiento de la ataxia espinocerebelosa
Swedish	Trehalos	Behandling av spinocerebellär ataxi
Norwegian	Trehalose	Behandling av spinocerebellar ataksi
Icelandic	Trehalósi	Meðferð spinocerebellar hreyfitruflunum

[1] At the time of designation