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EMA/COMP/785809/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ceftriaxone for the treatment of spinocerebellar ataxia

On 15 January 2015, orphan designation (EU/3/14/1425) was granted by the European Commission to Ospedale San Raffaele s.r.l., Italy, for ceftriaxone 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 subtypes of spinocerebellar ataxia, and depending on the subtype, 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 due to a progressive slow 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 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 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 511,100,000 (Eurostat 2014).

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How is this medicine expected to work?

This medicine is a type of antibiotic already used to treat certain infections. However, in spinocerebellar ataxia, ceftriaxone is expected to work by increasing the production of a protein called 'glutamate transporter' (GLT1), which regulates the amount of the substance glutamate in the brain. Too much glutamate in the brain is thought to contribute to the disease. By maintaining appropriate glutamate levels in the brain, ceftriaxone is expected to help reduce the symptoms of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of ceftriaxone in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with ceftriaxone in patients with spinocerebellar ataxia had been started.

At the time of submission, ceftriaxone was authorised in several EU countries for the treatment of certain bacterial infections, but it was not authorised anywhere in the EU for spinocerebellar ataxia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 December 2014 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

Sponsor's contact details:

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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	Ceftriaxone	Treatment of spinocerebellar ataxia
Bulgarian	Цефтриаксон	Лечение на спиноцеребеларна атаксия
Croatian	Ceftriakson	Liječenje spinocerebelarne ataksije
Czech	Ceftriaxon	Léčba spinocerebelárního ataxie
Danish	Ceftriaxon	Behandling af spinocerebellar ataksi
Dutch	Ceftriaxone	Behandeling van spinocerebellaire ataxie
Estonian	Tseftriaksoon	Spinotserebellaarataksia ravi
Finnish	Keftriaksoni	Spinoserebellaarisen ataksian hoito
French	Ceftriaxone	Traitement de l'ataxie spinocérébelleuse
German	Ceftriaxon	Behandlung der spinozerebellären Ataxie
Greek	Κεφτριαξόνη	Θεραπεία της νωτιαιοπαρεγκεφαλιδικής αταξίας
Hungarian	Ceftriaxon	Spinocerebelláris ataxia kezelése
Italian	Ceftriaxone	Trattamento dell'atassia spinocerebellare
Latvian	Ceftriaksons	Spinocerebellārās ataksijas ārstēšana
Lithuanian	Ceftriaksonas	Spinocerebeliarinės ataksijos gydymas
Maltese	Ceftriaxone	Kura tal-atassja spinoċerebellari
Polish	Ceftriakson	Leczenie ataksji rdzeniowo-móżdżkowej
Portuguese	Ceftriaxona	Tratamento de ataxia espinocerebelar
Romanian	Ceftriaxona	Tratamentul ataxiei spinocerebelare
Slovak	Ceftriaxón	Liečba spinocerebelárnej ataxie
Slovenian	Ceftriakson	Zdravljenje spinocerebelarne ataksije
Spanish	Ceftriaxona	Tratamiento de la ataxia espinocerebelosa
Swedish	Ceftriaxon	Behandling av spinocerebellär ataxi
Norwegian	Ceftriakson	Behandling av spinocerebellar ataksi
Icelandic	Ceftríaxón	Meðferð spinocerebellar hreyfitruflunum

[1] At the time of designation