8 March 2017
EMA/15858/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Trans-resveratrol for the treatment of spinocerebellar ataxia

On 12 January 2017, orphan designation (EU/3/16/1827) was granted by the European Commission to Luis Pereira de Almeida, Portugal, for trans-resveratrol for the treatment of spinocerebellar ataxia.

What is spinocerebellar ataxia?

Spinocerebellar ataxia is a group of genetic conditions of the nervous system whereby cells in certain areas of the brain get damaged and die. This leads to progressive problems with movement, coordination and balance (ataxia). Depending on the type of spinocerebellar ataxia, 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. Symptoms usually appear during adulthood.

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 condition.

[*]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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

The exact way this medicine works in spinocerebellar ataxia is not fully understood, but it is thought to activate an enzyme called sirtuin-1 (SIRT1), which is thought to play a role in protecting and preserving nerve cells. Activating SIRT1 is expected to help nerve cells to function normally and survive for longer, thus helping to relieve the symptoms of spinocerebellar ataxia.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine 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 8 December 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Trans-resveratrol	Treatment of spinocerebellar ataxia
Bulgarian	Транс-ресвератрол	Лечение на спиноцеребеларна атаксия
Croatian	Trans-resveratrol	Liječenje spinocerebelarne ataksije
Czech	Trans-resveratrol	Léčba spinocerebelárního ataxie
Danish	Trans-resveratrol	Behandling af spinocerebellar ataksi
Dutch	Trans-resveratrol	Behandeling van spinocerebellaire ataxie
Estonian	Trans-resveratrool	Spinotserebellaarataksia ravi
Finnish	Trans-resveratroli	Spinoserebellaarisen ataksian hoito
French	Trans-resveratrol	Traitement de l'ataxie spinocérébelleuse
German	Trans-resveratrol	Behandlung der spinozerebellären Ataxie
Greek	Trans- ρεσβερατρόλη	Θεραπεία της νωτιαιοπαρεγκεφαλιδικής αταξίας
Hungarian	Trans-resveratrol	Spinocerebelláris ataxia kezelése
Italian	Trans-resveratrolo	Trattamento dell'atassia spinocerebellare
Latvian	Trans-resveratrols	Spinocerebellārās ataksijas ārstēšana
Lithuanian	Trans-resveratrolis	Spinocerebeliarinės ataksijos gydymas
Maltese	Transresveratrol	Kura tal-atassja spinoċerebellari
Polish	Trans-resweratrol	Leczenie ataksji rdzeniowo-móżdżkowej
Portuguese	Trans-resveratrol	Tratamento de ataxia espinocerebelar
Romanian	Trans-resveratrol	Tratamentul ataxiei spinocerebelare
Slovak	Trans-resveratrol	Liečba spinocerebelárnej ataxie
Slovenian	Trans-resveratrol	Zdravljenje spinocerebelarne ataksije
Spanish	Trans-resveratrol	Tratamiento de la ataxia espinocerebelosa
Swedish	Trans-resveratrol	Behandling av spinocerebellär ataxi
Norwegian	Trans-resveratrol	Behandling av spinocerebellar ataksi
Icelandic	Trans-resveratról	Meðferð spinocerebellar hreyfitruflunum

[1] At the time of designation