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Public summary of opinion on orphan designation

Acetylleucine for the treatment of spinocerebellar ataxia

On 22 November 2018, orphan designation (EU/3/18/2059) was granted by the European Commission to IntraBio Ltd, United Kingdom, for acetylleucine 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 including physiotherapy, occupational therapy, and speech therapy 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 517,400,000 (Eurostat 2018).

How is this medicine expected to work?

Acetylleucine has been authorised in some countries for the treatment of vertigo (a spinning sensation that affects balance). The way acetylleucine works in spinocerebellar ataxia is not clear, but it is thought to stabilise the nerve cells responsible for balance and for coordinating movement. This is expected to improve movement control and coordination in patients with the disease.

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 were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2018 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

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Acetyllecine	Treatment of spinocerebellar ataxia
Bulgarian	Ацетиллевцин	Лечение на спиноцеребеларна атаксия
Croatian	Acetilleucin	Liječenje spinocerebelarne ataksije
Czech	Acetyllecine	Léčba spinocerebelárního ataxie
Danish	Acetyllecine	Behandling af spinocerebellar ataksi
Dutch	Acetyllecine	Behandeling van spinocerebellaire ataxie
Estonian	Atsetülletsiin	Spinotserbellaarataksia ravi
Finnish	Asetyylileusiini	Spinotserbellaarisen ataksian hoito
French	Acétyllecine	Traitement de l'ataxie spinocérébelleuse
German	Acetyllecine	Behandlung der spinocerebellären Ataxie
Greek	Ακετυλλευκίνη	Θεραπεία της νωτιαιοπαρεγκεφαλιδικής αταξίας
Hungarian	Acetilleucin	Spinocerebelláris ataxia kezelése
Italian	Acetilleucina	Trattamento dell'ataxia spinocerebellare
Latvian	Acetilleicīns	Spinocerebellārās ataksijas ārstēšana
Lithuanian	Acetilleucinas	Spinocerebeliarinės ataksijos gydymas
Maltese	Acetyllecine	Kura tal-atassja spinocerebellari
Polish	Acetyloleucyna	Leczenie ataksji rdzeniowo-mózdzkowej
Portuguese	Acetilleucina	Tratamento de ataxia espinocerebelar
Romanian	Acetilleucină	Tratamentul ataxiei spinocerebelare
Slovak	Acetyllecín	Liečba spinocerebelárnej ataxie
Slovenian	Acetillevcin	Zdravljenje spinocerebelarne ataksije
Spanish	Acetileucina	Tratamiento de la ataxia espinocerebelosa
Swedish	Acetyllecine	Behandling av spinocerebellär ataxi
Norwegian	Acetyllecine	Behandling av spinocerebellar ataksi
Icelandic	Asetýllecín	Meðferð spinocerebellar hreyfitruflunum

¹ At the time of designation