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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium valproate for the treatment of 5q spinal muscular atrophy

First publication	29 November 2005
Rev.1: withdrawal from the Community Register	16 December 2011
Rev.2: administrative update	16 December 2013

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in December 2011 on request of the sponsor.

On 26 August 2005, orphan designation (EU/3/05/309) was granted by the European Commission to Jennifer Trust for Spinal Muscular Atrophy, United Kingdom, for sodium valproate for the treatment of 5q spinal muscular atrophy.

What is 5q spinal muscular atrophy?

5q spinal muscular atrophy (SMA) is an inherited disease. The abnormal gene is located on the long arm (q arm) of chromosome 5 (hence the name “5q”) and the disease occurs if both parents pass this gene onto their offspring (called autosomal recessive transmission). Due to this defect a structure called spinal motor neuron (SMN) protein is lacking. Normally SMN protein is essential to the normal functioning and survival of the nervous cells responsible for muscular activity (motor neurons). Without this protein these neurons may deteriorate and eventually die, resulting in muscle weakness.

In 5q SMA muscle weakness is found most often at the level of the proximal muscles (the muscles closest to the trunk), but in the most severe cases respiratory muscles can be affected too leading to increased risk of lung infections and breathing problems. The disease is life-threatening and chronically debilitating.

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

At the time of designation, 5q spinal muscular atrophy affected not more than 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 14,000 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?

No satisfactory methods exist that were authorised at the time of application.
Several treatment methods consisting of medicines or non-medicinal approaches have been tried or are under investigation

How is this medicine expected to work?

Sodium valproate is a medicine currently used to control the convulsions (fits or seizures) in some forms of epilepsy. Although the exact mechanism of action in 5q spinal muscular atrophy is not known, it is suggested that sodium valproate would increase the production of the missing SMN protein. This might result in improving the functioning of the nerves, affected in patients with 5q spinal muscular atrophy.

What is the stage of development of this medicine?

The evaluation of the effects of sodium valproate in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with 5q spinal muscular atrophy were initiated.

Sodium valproate was not marketed anywhere worldwide for the treatment of 5q spinal muscular atrophy or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2005 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.

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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 official EU languages[1], Norwegian and Icelandic.

Language	Active Ingredient	Indication
English	Sodium valproate	Treatment of 5q spinal muscular atrophy
Czech	Valproát sodný	Léčba 5q spinální muskulární atrofie
Danish	Natriumvalproat	Behandling af 5q spinal muskelatrofi
Dutch	Natriumvalproaat	Behandeling van 5q spinale spieratrofie
Estonian	Naatriumvalproaat	5q spinaalse lihasatroofia ravi
Finnish	Natriumvalproaatti	Selkärangan 5q lihassurkastuman hoito
French	Valproate de sodium	Traitement de l'amyotrophie spinale 5q
German	Natriumvalproat	Behandlung der 5q spinalen Muskelatrophie
Greek	βαλπροϊκό νάτριο	Θεραπεία της νωτιαίας μυϊκής ατροφίας (5q)
Hungarian	Nátrium valproat	5q spinális izomatrophia kezelése
Italian	Valproato di sodio	Trattamento dell'atrofia muscolare spinale 5q
Latvian	Nātrija valproāts	5q spinālas muskuļu atrofijas ārstēšana
Lithuanian	Natrio valproatas	Nugaros raumenų atrofijos gydymas, esant 5q srities delecijoms
Polish	Sodu walproinian	Leczenie rdzeniowego zaniku mięśni 5q
Portuguese	Valproato de sódio	Tratamento da atrofia muscular espinal 5q
Slovak	Natrii valproas	Liečba 5q spinálnej svalovej atrofie
Slovenian	Natrijev valproat	Zdravljenje 5q spinalne mišične atrofije
Spanish	Valproato sódico	Tratamiento de la atrofia muscular espinal 5q
Swedish	Natriumvalproat	Behandling av 5q spinal muskelatrofi
Norwegian	Natriumvalproat	Behandling av 5q spinal muskelatrofi
Icelandic	Natríum valpróat	Meðferð við 5q mænuvöðvarýrnunar

[1] At the time of designation