European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF (2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide for the treatment of progressive myoclonic epilepsies

On 26 August 2005, orphan designation (EU/3/05/315) was granted by the European Commission to UCB SA, Belgium, for (2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide for the treatment of progressive myoclonic epilepsies.
The sponsorship was transferred to UCB Pharma SA, Belgium, in July 2007.

What are progressive myoclonic epilepsies?

Progressive myoclonic epilepsies are a group of 6 inherited diseases, with an onset in mid to late childhood. They are due to an imbalance in the brain's electrical activity. This imbalance causes seizures that in the affected children develop to myoclonic seizures associated with clonic seizures. Myoclonic seizures are sudden and brief muscle contractions that may involve one part of the body or the entire body. Clonic seizures are a form of movement marked by involuntary contractions and relaxations of a muscle, occurring in rapid succession. The mental state of the patient deteriorates after subsequent episodes of attacks, leading to delayed or hindered psychomotor development, with delayed skill acquisitions or regression. Progressive myoclonic epilepsies are considered as chronically debilitating and life threatening condition.

What are the methods of treatment available?

Antiepileptic drugs are currently used for the symptomatic treatment of the epileptic seizures of the condition. Several medicinal products were authorised for the condition in the Community, at the time of submission of the application for the orphan drug designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that (2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide might be of potential significant benefit for the treatment of progressive myoclonic epilepsies mainly because it might improve the long-term outcome of the patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, progressive myoclonic epilepsies was considered to affect not more than 0.5 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 23,000 persons.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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How is this medicinal product expected to act?
(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] is a chemical substance that might have an anticonvulsant effect. An anticonvulsant acts on the brain to prevent and reduce the frequency and severity of seizures attacks.

What is the stage of development of this medicinal product?
The evaluation of the effects of (2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with progressive myoclonic epilepsies were initiated.

(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide was not authorised anywhere worldwide for progressive myoclonic epilepsies or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 13 July 2005 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

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Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic

Language	Active Ingredient	Indication
English	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide	Treatment of progressive myoclonic epilepsies
Czech	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Léčba progresivní myoklonické epilepsie
Danish	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Behandling af progressive myoklone epilepsier
Dutch	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamide	Behandeling van progressieve myoklonische epilepsie
Estonian	(2S)-2-[(4R)-2-oksü-4-propüültetrahüdro-1H-pürrool-1-yl] butanamiid	Progresseeruva müokloonilise epilepsia ravi
Finnish	(2S)-2-[(4R)-2-oksi-4-propyylitetrahydro-1H-pyrroli-1-yl] butanamidi	Etenevien myoklonusepilepsioiden hoito
French	(2S)-2-((4R)-2-oxo-propylpyrrolidine-1-yl) butanamide	Traitement des épilepsies myocloniques progressives
German	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Behandlung der progressiven myoklonischen Epilepsie
Greek	(2S)-2-[(4R)-2-οξο-4-προπυλοτετραϋδρο-1H-πυρρολ-1-υλ] βουταναμίδιο	Θεραπεία της προοδευτικής μυοκλονικής επιληψίας
Hungarian	(2S)-2-[(4R)-2-oxo-4-propiltetrahidro-1H-pirrol-1-il] butánamid	Progresszív myoclonusos epilepszia kezelése
Italian	(2S)-2-[(4R)-2-oxo-4-propiltetraidro-1H-pirrol-1-il] butanammide	Trattamento delle epilessie miocloniche progressive
Latvian	(2S)-2-[(4R)-2-okso-4-propiltetrahidro-1H-pirol-1-il] butanamīds	Progresējošas miokloniskas epilepsijas ārstēšanai
Lithuanian	(2S)-2-[(4R)-2-okso-4-propiltetrahidro-1H-pyrol-1-yl] butanamidas	Progresuojančios miokloninės epilepsijos gydymas
Polish	(2S)-2-[(4R)-2-okso-4-propyltetrahydro-1H-pirol-1-yl] butanamid	Leczenie padaczek mioklonicznych postępujących
Portuguese	(2S)-2-[(4R)-2-oxo-4-propiltetrahidro-1H-pirrol-1-il] butanamida	Tratamento de epilepsias mioclónicas progressivas
Slovak	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Liečba progresívne myoklonickej epilepsie
Slovenian	(2S)-2-[(4R)-2-okso-4-propiltetrahidro-1H-pirol-1-il] butanamid	Zdravljenje progresivne miokloničnе epilepsije
Spanish	(2S)-2-[(4R)-2-oxo-4-propiltetrahidro-1H-pirrol-1-il] butanamida	Tratamiento de epilépsias mioclónicas progresivas
Swedish	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Behandling av progressiva myokloniska epilepsier
Norwegian	(2S)-2-[(4R)-2-oxo-4-propyltetrahydro-1H-pyrrol-1-yl] butanamid	Behandling av progressive myoklone epilepsier
Icelandic	(2S)-2-[(4R)-2-oxó-4-própýltetrahýdró-1H-pýrról-1-ýl] bútanamíð	Meðferð á versnandi vöðvakippaflogum

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