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 stiripentol for the treatment of severe myoclonic epilepsy in infancy

On 5 December 2001 orphan designation (EU/3/01/071) was granted by the European Commission to Biocodex, France, for stiripentol for the treatment of severe myoclonic epilepsy in infancy.

What is severe myoclonic epilepsy in infancy?

Severe myoclonic epilepsy in infancy is a disease of early childhood. The onset is in the first year of life where affected infants develop clonic seizures or fits. Clonic seizures are a form of movement marked by involuntary contractions and relaxations of a muscle, occurring in rapid succession. They are due to an imbalance in the electrical activity of the brain. Later, usually more than one year after onset, myoclonic jerks appear. These attacks are sudden and brief muscle contractions that may involve one part of the body or the entire body. The mental state of the child deteriorates after subsequent episodes of attacks, leading to delayed or hindered psychomotor development, with delayed skill acquisitions or regression. Severe myoclonic epilepsy in infancy is 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. At the time of submission of the application for the orphan drug designation there were medicinal products authorised for the condition in the Community.
Satisfactory argumentation has been submitted by the sponsor to justify the assumption that stiripentol might be of potential significant benefit for the treatment of severe myoclonic epilepsy in infancy 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**?

According to the information provided by the sponsor, severe myoclonic epilepsy in infancy was considered to affect about 15,000 persons in the European Union.

How is this medicinal product expected to act?

Stiripentol 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 seizure attacks. The mechanism of action is not yet completely understood.

* Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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What is the stage of development of this medicinal product?
The effects of stiripentol were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with severe myoclonic epilepsy in infancy were ongoing.

Stiripentol was not marketed anywhere worldwide for the treatment of severe myoclonic epilepsy in infancy 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 7 September 2001 a positive opinion recommending the grant of the above-mentioned designation.

Update: Stiripentol (Diacomit) is authorised in the European Union as of 4 January 2007 for use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with severe myoclonic epilepsy in infancy (SMEI or Dravet's syndrome) whose seizures are not adequately controlled with clobazam and valproate.
For more information please see www.emea.europa.eu

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

Language	Active Ingredient	Indication
English	stiripentol	Treatment of severe myoclonic epilepsy in infancy
Danish	stiripentol	Behandling af svær myoklonisk epilepsi hos spædbørn.
Dutch	stiripentol	Behandeling van ernstige infantiele myoklonische epilepsie.
Finnish	stiripentoli	Lapsuusiän vaikean myoklonisen epilepsian hoito
French	stiripentol	Traitement de l'épilepsie myoclonique sévère du nourrisson
German	Stiripentol	Behandlung schwerer myoklonischer Epilepsien im Kleinkindesalter
Greek	stiripentol	Θεραπεία σοβαρής μυοκλονικής επιληψίας στη βρεφική ηλικία
Italian	stiripentol	Trattamento dell'epilessia mioclonica grave dell'infanzia
Portuguese	stiripentol	Tratamento da epilepsia mioclónica grave na primeira infância
Spanish	estiripentol	Tratamiento de la epilepsia mioclónica grave en la infancia
Swedish	stiripentol	Behandling av svår myoklon epilepsi hos spädbarn