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

Document Date: London, 28 February 2007
Doc.Ref.: EMEA/122307/2004 Rev.1

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF rufinamide for the treatment of Lennox-Gastaut syndrome

On 20 October 2004, orphan designation (EU/3/04/240) was granted by the European Commission to Eisai Limited, United Kingdom, for rufinamide for the treatment of Lennox-Gastaut syndrome.

What is Lennox-Gastaut syndrome?
Lennox-Gastaut syndrome is a severe childhood epilepsy syndrome. Seizures usually start between 2 and 5 years of age, but may start under two years. Seizure types, which vary among patients, include tonic (stiffening of the body, upward deviation of the eyes, dilation of the pupils, and altered respiratory patterns), atonic (brief loss of muscle tone and consciousness, causing abrupt falls), atypical absence (staring spells), and myoclonic (sudden muscle jerks). Most children with Lennox-Gastaut syndrome experience some degree of impaired intellectual functioning or information processing, along with developmental delays, and behavioural disturbances. Severe myoclonic epilepsy in infancy is considered a chronically debilitating condition.

What are the methods of treatment available?
Several products with anti-epileptic activity were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Some children need more than one anti-epileptic medicine and there are many children for whom no anti-epileptics are effective.
Rufinamide, when used in combination with other anti-epileptic medicine, might be of potential significant benefit for the treatment of Lennox-Gastaut syndrome. Rufinamide might help to achieve better control over the frequency of seizures. These benefits 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, Lennox-Gastaut syndrome was considered to affect between 46,000 to 92,000 persons in the European Union.

How is this medicinal product expected to act?
The precise mechanism of action of rufinamide is unknown, however, studies in experimental models suggest that it may act on neurons (cells in the nervous system) to block the spread of seizure activity.

What is the stage of development of this medicinal product?
The effects of rufinamide were evaluated in experimental models. At the time of submission of the application for orphan designation, a clinical trial in patients with Lennox-Gastaut syndrome was completed.

Rufinamide was not marketed anywhere worldwide for Lennox-Gastaut syndrome at the time of submission. Orphan designation of rufinamide was granted in the United States for Lennox-Gastaut syndrome on 8 October 2004.

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.eu.int www.emea.eu.int

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 9 September 2004 a positive opinion recommending the grant of the above-mentioned designation.

Update: Rufinamide (Inovelon) is authorised in the European Union as of 16 January 2007 for adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients 4 years and older.
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.

For more information:
Sponsor's contact details:
Eisai Limited
3, Shortlands,
London, W6 8EE
United Kingdom
Telephone: (44-20) 86 00 14 28
Telefax: (44-20) 86 00 14 82
E-mail: Imedinfo@eisai.net

*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.

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

Language	Active Ingredient	Indication
English	Rufinamide	Treatment of Lennox-Gastaut syndrome
Czech	Rufinamid	Léčba Lennox-Gastautova syndromu
Danish	Rufinamid	Behandling af Lennox-Gastaut syndrom
Dutch	Rufinamide	Behandeling van het Lennox-Gastaut syndroom
Estonian	Rufinamiid	Lennox-Gastaut´ sündroomi raviks
Finnish	Rufinamidi	Lennox-Gastautin oireyhtymän hoito
French	Rufinamide	Traitement du syndrome de Lennox-Gastaut
German	Rufinamid	Behandlung des Lennox-Gastaut-Syndroms
Greek	Ρουφιναμίδη	θεραπεία του Συνδρόμου Lennox-Gastaut
Hungarian	Rufinamid	Lennox-Gastaut szindróma kezelése
Italian	Rufinamide	Trattamento della sindrome di Lennox-Gastaut
Latvian	Rufinamīds	Lenoksa – Gasto sindroma terapija
Lithuanian	Rufinamidas	Lenokso – Gasto sindromo gydymas
Maltese	Rufinamid	Treatment of Lennox-Gastaut syndrome
Polish	Rufinamid	Leczenie zespołu Lennox-Gastauta
Portuguese	Rufinamida	Tratamento do síndrome de Lennox-Gastaut
Slovak	Rufinamid	Liečba Lennox-Gastautovho syndrómu
Slovenian	Rufinamid	Zdravljenje Lennox-Gastautovega sindroma
Spanish	Rufinamida	Tratamiento del síndrome de Lennox-Gastaut
Swedish	Rufinamid	Behandling av Lennox-Gastaut syndrom
Norwegian	Rufinamid	Behandling av Lennox-Gastaut syndromet
Icelandic	Rufinamíð	Meðferð á Lennox-Gastaut heilkenni