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 ethyl eicosopentaenoate for the treatment of Huntington's disease

On 29 December 2000, orphan designation (EU/3/00/013) was granted by the European Commission to Laxdale Ltd., United Kingdom, for ethyl eicosopentaenoate for the treatment of Huntington's disease. The name of the sponsor has since changed to Amarin Neuroscience Ltd.

What is Huntington's disease?
Huntington's disease is a hereditary disease where the cells of the nervous system (neurons) degenerate. The neurons produce a group of substances, the so-called neurotransmitters that are released when the neuron is activated. These neurotransmitters will then activate or inhibit the target cell. In Huntington's disease, due to the extensive degeneration of neurons, the nervous system cannot regulate properly the functioning of the target organs anymore. As a consequence, the disease is characterised by involuntary movements, behavioural disturbances and mental deterioration. The disease progresses over time and is chronically debilitating with potentially life-threatening complications.

What are the methods of treatment available?
No satisfactory methods exist that were authorised at the time of application.

What is the estimated number of patients affected by the condition*?
According to the information provided by the sponsor, Huntington's disease was considered to affect between 15,000 and 26,000 persons in the European Union.

How is this medicinal product expected to act?
Enzymes are proteins produced by the human body that speed up the transformation of certain substances into other substances. One of the possible causes of Huntington's disease might be the activation of a certain enzyme, phospholipase A_2 contributing to the degeneration of the neurons. Although the mechanism of action of ethyl eicosopentaenoate in Huntington's disease is not known, it appears to inhibit the activity of this enzyme phospholipase A_2. This might slow down the disease progression to a certain extent, but it is not expected that it will have any curative effect.

What is the stage of development of this medicinal product?
The effects of ethyl eicosopentaenoate were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with Huntington's disease were ongoing.
Ethyl eicosopentaenoate was authorised in Japan for treatment for peripheral vascular disease and elevated triglycerides at the time of submission. Orphan designation of ethyl eicosopentaenoate was granted in the United States for treatment of Huntington's disease.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 27 October 2000 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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*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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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Ethyl Eicosopentaenoate	Treatment of Huntington's disease
Danish	eicosapentaenoat (-) aetyl	Behandling af Huntington's sygdom
Dutch	Ethyl-eicosapentanoaat	Behandeling van de ziekte van Huntington
Finnish	etyylieikosapentaenoaatti	Huntingtonin taudin hoito
French	Eicosapentaenoate d'éthyle	Traitement de la maladie d'Huntington
German	ethyl-eicosapentaenoat	Behandlung der Huntington Erkrankung
Greek	εικοσιπεντανοϊκός αιθυλεστέρας	Θεραπεία της νοσου Huntington
Italian	etil-eicosapentaenoato	Trattamento della malattia di Huntington
Portuguese	Etil-eicosapentanoato	Tratamento da doença de Huntington
Spanish	Eicosapentaenoato de etilo	Tratamiento de la enfermedad de Huntington
Swedish	etyleikosapentanoat	Behandling av Huntingtons sjukdom