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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ethanol (96 per cent) (gel for injection) for the treatment of congenital lymphatic malformations

On 8 March 2004, orphan designation (EU/3/04/190) was granted by the European Commission to Orfagen, France, for ethanol (96 per cent) (gel for injection) for the treatment of congenital lymphatic malformations.

What are congenital lymphatic malformations?

Congenital lymphatic malformations are the result of an abnormal development of lymphatic vessels that starts before birth. It results in an alteration of the structure of the lymphatic network in a particular area of the body. These vascular lesions can include small birthmarks to deforming lesions. Congenital lymphatic malformations can cause body image problems, pain, and depending on their localisation distortion of some normal functions. In extreme cases, particularly when the airways are obstructed by the malformation, the condition can be life threatening due to the impairment of vital functions.

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

At the time of designation, congenital lymphatic malformations affected approximately 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 39,000 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. Surgery is an option for some lesions but the success of the intervention is limited as it depends on the characteristics of the lesion (localisation, size, involvement of neighbouring structures, etc.).

[*]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 385,000,000 (Eurostat 2002) 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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

How is this medicine expected to work?

Ethanol (96 per cent) (gel for injection) can be injected into the lymphatic vessels where it acts on the proteins destroying their structure and triggering their clotting. These clots (also known as emboli) can obstruct vessels and then reduce the volume of the malformation, acting as an embolising agent. In addition, ethanol has a direct effect on the internal surface on the vessels, as it damages the cells that form the internal layer of the vessels. This effect destroys the vessel wall and results on its occlusion (sclerosis). Both actions combined could have as consequence the destruction of the abnormal vessels that constitute the lesions.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with congenital lymphatic malformations were ongoing.

Ethanol (96 per cent) (gel for injection) was not marketed anywhere worldwide for congenital lymphatic malformations 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 5 February 2004 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.

For more information

Sponsor's contact details:

Orfagen
CRDPF Langlade
3 Avenue Hubert Curien - BP 13562
31035 Toulouse Cedex 1
France
Telephone: +33 5 34 50 64 58
Telefax: +33 5 34 50 34 57
E-mail: info@orfagen.com

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 all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Ethanol (96 per cent) (gel for injection)	Treatment of congenital lymphatic malformations
Danish	Ethanol (96 procent) (injektionsgel)	Behandling af kongenital misdannelse i lymfesystemet
Dutch	Ethanol (96 percent) (gel voor injectie)	Behandeling van congenitale lymfatische misvormingen
Finnish	Etanoli (96 prosenttia) (injektiogeeli)	Synnynnäisten imusuonten epämuodostumien hoito
French	Ethanol (96 pour cent) (gel pour injection)	Traitement des malformations lymphatiques congénitales
German	Ethanol (96 Prozent) (Gel für Injektion)	Behandlung der kongenitalen lymphatischen Missbildungen
Greek	Αιθανόλη (96 τοις εκατό) (ενέσιμη γέλη)	Θεραπεία συγγενών δυσπλασιών λεμφικού συστήματος
Italian	Etanolo (96 percento) (gel iniettabile)	Trattamento delle malformazioni congenite linfatiche
Portuguese	Etanol (96 por cento) (gel injectável)	Tratamento das malformações linfáticas congénitas
Spanish	Etanol (96 por ciento) (gel inyectable)	Tratamiento de las malformaciones linfáticas congénitas
Swedish	Etanol (96 procent) (gel för injektion)	Behandling av kongenitala lymfatiska missbildningar

[1] At the time of designation