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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 venous malformations

On 8 March 2004, orphan designation (EU/3/04/191) was granted by the European Commission to Orfagen, France, for ethanol (96 per cent) (gel for injection) for the treatment of congenital venous malformations.

What are congenital venous malformations?

Congenital venous malformations are the result of abnormal development of veins that starts before birth. It results in an alteration of the structure of the venous network in a particular area of the body. These vascular lesions can include from small birthmarks to deforming lesions. Congenital vascular malformations can cause body image problems, pain, and depending on its 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 venous malformations affected approximately 4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 154,000 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.

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How is this medicine expected to work?

Ethanol (96 per cent) (gel for injection) can be injected into the malformed veins where it acts on the blood proteins destroying its structure and triggering their clotting. These clots (also known as emboli) can obstruct veins 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 veins, as 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 venous malformations were ongoing.

Ethanol (96 per cent) (gel for injection) was not marketed anywhere worldwide for congenital venous 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:

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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 venous malformations
Danish	Ethanol (96 procent) (injektionsgel)	Behandling af kongenital venøs misdannelse
Dutch	Ethanol (96 percent) (gel voor injectie)	Behandeling van congenitale veneuze misvormingen
Finnish	Etanoli (96 prosenttia) (injektiogeeli)	Synnynnäisten laskimoepämuodostumien hoito
French	Ethanol (96 pour cent) (gel pour injection)	Traitement des malformations veineuses congénitales
German	Ethanol (96 Prozent) (Gel für Injektion)	Therapie der kongenitalen venösen Missbildungen
Greek	Αιθανόλη (96 τοις εκατό) (ενέσιμη γέλη)	Θεραπεία συγγενών φλεβικών δυσπλασιών
Italian	Etanolo (96 percento) (gel iniettabile)	Trattamento delle malformazioni congenite venose
Portuguese	Etanol (96 por cento) (gel injectável)	Tratamento de malformações venosas congénitas
Spanish	Etanol (96 por ciento) (gel inyectable)	Tratamiento de malformaciones venosas congénitas
Swedish	Etanol (96 procent) (gel för injektion)	Behandling av kongenitala venösa missbildningar

[1] At the time of designation