



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation tacrolimus hydrate for the treatment of vernal keratoconjunctivitis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2010 on request of the sponsor.

On 14 January 2004, orphan designation (EU/3/03/185) was granted by the European Commission to Sucampo Pharma Europe Ltd., United Kingdom, for tacrolimus hydrate for the treatment of vernal keratoconjunctivitis.

What is vernal keratoconjunctivitis?

Vernal keratoconjunctivitis (also known as spring catarrh) is a chronic allergic inflammation of the conjunctiva (membrane lining the eyelid and covering the eyeball) and the cornea (clear tissue in the front of the eye that protects deeper structures), most often seen in young children and with seasonal appearance. This inflammation leads to redness, fluid discharge, itch, pain and light sensitivity.

Vernal keratoconjunctivitis is often seen together with other allergic conditions (e.g. eczema and asthma) and is chronically debilitating due to possible corneal ulcers and sight loss.

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

At the time of designation, vernal keratoconjunctivitis affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 36,000 people, and is below the threshold 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?

Various medicinal products were authorised for the treatment of vernal keratoconjunctivitis in the Community at the time of submission of the application for orphan drug designation.

*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.



Tacrolimus hydrate might be of potential significant benefit for the treatment of vernal keratoconjunctivitis. Tacrolimus hydrate eye drops might help to achieve better overall control of the condition. These benefits will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Tacrolimus hydrate is an immunomodulator, in other words it acts by inhibiting the cells from the defence system involved in the inflammation process.

What is the stage of development of this medicine?

The effects of tacrolimus hydrate eye drops were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with vernal keratoconjunctivitis were ongoing.

The medicinal product was not marketed anywhere worldwide for vernal keratoconjunctivitis 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 December 2003 recommending the granting of this designation.

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 European Union) 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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Patient associations' contact points

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Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Tacrolimus hydrate	Treatment of vernal keratoconjunctivitis
Danish	Tacrolimushydrat	Behandling af keratoconjunctivitis vernalis
Dutch	Tacrolimushydraat	Behandeling van vernale keratoconjunctivitis
Finnish	Takrolimuusihydraatti	Vernaalisen keratokonjunktiviitin hoito
French	Hydrate de tacrolimus	Traitement de la kératoconjunctivite vernale
German	Tacrolimushydrat	Behandlung der Keratoconjunctivitis vernalis
Greek	τακρολίμους -ένυδρος	Θεραπεία της εαρινής κερατοεπιπεφυκίτιδας
Italian	Tacrolimus idrato	Trattamento della cheratocongiuntivite primaverile
Portuguese	Hidrato de tacrolimus	Tratamento da queratoconjuntivite vernal
Spanish	Hidrato de tacrolimús	Tratamiento de la queratoconjuntivitis vernal
Swedish	Takrolimushydrat	Behandling av vernal keratokonjunktivit

¹ At the time of designation