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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Ciclosporin for the vernal keratoconjunctivitis

First publication	23 February 2009
Rev.1: administrative update	25 May 2011
Rev.2: sponsor's name change	24 April 2015
<b>Disclaimer</b> Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 6 April 2006, orphan designation (EU/3/06/360) was granted by the European Commission to Novagali Pharma, France, for ciclosporin for the treatment of vernal keratoconjunctivitis.

In March 2015, Novagali Pharma SA changed name to SANTEN SAS.

#### What is vernal keratoconjunctivitis?

Vernal keratoconjunctivitis (also known as spring catarrh) is a chronic allergic inflammation (swelling and redness) 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, itching, pain and light sensitivity.

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

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

At the time of designation, vernal keratoconjunctivitis affected between 1 to 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 47,000 to 141,000 people<sup>\*</sup>, and is below

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<sup>\*</sup>Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).



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?**

Some 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.

Satisfactory argumentation has been submitted by the sponsor to justify that ciclosporin might be of potential significant benefit for the treatment of vernal keratoconjunctivitis because it may bring better control of the symptoms 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?**

Ciclosporin is an immunosuppressant drug; it inhibits the function of the cells of the immune system (body's defence to infection and disease). In patients with vernal keratoconjunctivitis it is expected to prevent the inflammation observed in conjunctiva and cornea.

### **What is the stage of development of this medicine?**

The effects of ciclosporin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with vernal keratoconjunctivitis were ongoing.

Ciclosporin was not authorised 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 8 March 2006 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<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Ciclosporin	Treatment of vernal keratoconjunctivitis
Czech	Ciclosporin	Léčení vernální keratokonjunktividy
Danish	Ciclosporin	Behandling af keratoconjunctivitis vernalis
Dutch	Ciclosporine	Behandeling van vernale keratoconjunctivitis
Estonian	Tsüklosporiin	Vernaalse keratokonjunktiviidi ravi
Finnish	Siklosporiini	Vernaalisen keratokonjunktiviitin hoito
French	Ciclosporine	Traitement de la kératoconjunctivite vernale
German	Ciclosporin	Behandlung der Keratoconjunctivitis vernalis
Greek	Κυκλοσπορίνη	Θεραπεία της εαρινής κερατοεπιπεφυκίτιδας
Hungarian	Cyclosporin	Tavaszi keratoconjunctivitis kezeléské
Italian	Ciclosporina	Trattamento della cheratocongiuntivite primaverile
Latvian	Ciklosporīns	Vernālā keratokonjunktivīta ārstēšana
Lithuanian	Ciklosporinas	Pavasarinio keratokonjunktivito gydymas
Polish	Cyklosporyna	Leczenie wiosennego zapalenia rogówki i spojówki
Portuguese	Ciclosporina	Tratamento da queratoconjuntivite vernal
Slovak	Cyklosporín	Liečba vernálnej keratokonjunktivitídy
Slovenian	Ciklosporin	Zdravljenje vernalnega keratokonjunktivitisa
Spanish	Ciclosporina	Tratamiento de la queratoconjuntivitis vernal
Swedish	Ciklosporin	Behandling av vernal keratokonjunktivit
Norwegian	Ciklosporin	Behandling av vernal keratokonjunktivitt
Icelandic	Ciklósporín	Meðferð hornhimnu og augnslímhúðarbólgu

<sup>1</sup> At the time of designation