



European Medicines Agency  
Pre-authorisation Evaluation of Medicines for Human Use

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*Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in November 2004 on request from the sponsor.*

## **COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

### **PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF ciclosporin for the treatment of atopic keratoconjunctivitis**

On 14 April 2004 orphan designation (EU/3/04/196) was granted by the European Commission to Allergan Pharmaceuticals Ireland, for ciclosporin for the treatment of atopic keratoconjunctivitis.

#### **What is atopic keratoconjunctivitis?**

Atopic keratoconjunctivitis is a chronic allergic inflammation of the conjunctiva (membrane lining the eyelid and covering the eyeball), the cornea (clear tissue in the front of the eye that protects deeper structures) and eyelids, which is associated with atopic dermatitis (eczema). A characteristic sign of atopic keratoconjunctivitis is eyelid skin that is red, scaly and weeping. The conjunctiva may also be swollen and red and may discharge clear fluid or pus due to inflammation. Unfortunately, this may persist for many years. Common symptoms of this long-term condition are severe photophobia (an aversion to bright light) and weeping and itching of the eyes.

Atopic keratoconjunctivitis is chronically debilitating due to possible corneal complications, cataract and sight loss.

#### **What are the methods of treatment available?**

Various medicinal products were available for the treatment of atopic keratoconjunctivitis and were authorised in some Member States in the Community at the time of submission of the application for orphan drug designation. Other potential therapies include non pharmacological approaches. Ciclosporin might be of potential significant benefit for the treatment of atopic keratoconjunctivitis. Ciclosporin 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.

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

According to the information provided by the sponsor, atopic keratoconjunctivitis was considered to affect about 77,000 persons in the European Union.

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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 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.

**How is this medicinal product expected to act?**

Ciclosporin 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 medicinal product?**

The effects of ciclosporin were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with atopic keratoconjunctivitis were ongoing.

Ciclosporin was not marketed anywhere worldwide for atopic keratoconjunctivitis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 8 March 2004 a positive opinion recommending the grant of the above-mentioned designation.

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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.

**For more information:**

Sponsor's contact details:

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### Translations of the active ingredient and indication in all EU languages

| Language   | Active Ingredient | Indication                                     |
|------------|-------------------|------------------------------------------------|
| English    | Ciclosporin       | Treatment of atopic keratoconjunctivitis       |
| Danish     | Ciclosporin       | Behandling af atopisk keratokonjunktivit       |
| Dutch      | Ciclosporine      | Behandeling van atopische keratoconjunctivitis |
| Finnish    | Siklosporiinia    | Atooppisen keratokonjunktiviitin hoito         |
| French     | Ciclosporine      | Traitement des kératoconjunctivites atopiques  |
| German     | Ciclosporin       | Behandlung der atopischen Keratokonjunktivitis |
| Greek      | Κυκλοσπορίνη      | Θεραπεία της ατοπικής κερατοεπιπεφυκτίδας      |
| Italian    | Ciclosporina      | Trattamento della cheratocongiuntivite atopica |
| Portuguese | Ciclosporina      | Tratamento da queratoconjuntivite atópica      |
| Spanish    | Ciclosporina      | Tratamiento de la queratoconjuntivitis atópica |
| Swedish    | Ciclosporin       | Behandling av atopisk keratokonjunktivit       |