



## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of ciclosporin (eye drops, solution) for the treatment of atopic keratoconjunctivitis**

On 24 July 2009, orphan designation (EU/3/09/651) was granted by the European Commission to Allergan Pharmaceuticals Ireland, Ireland, for ciclosporin (eye drops, solution) for the treatment of atopic keratoconjunctivitis.

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

Atopic keratoconjunctivitis is long-term inflammation of the eye that can occur in patients with atopic dermatitis (eczema). It causes the skin of the eyelids to become red and scaly, and to weep fluid. The conjunctiva (the membrane lining the eyelid and covering the eyeball) may also become swollen and red, sometimes with a discharge of clear fluid or pus. The inflammation can also affect the cornea (the transparent surface at the front of the eye that covers the iris and the pupil). Patients with this disease have itchy, painful eyes and blurred vision, and are oversensitive to light.

Atopic keratoconjunctivitis is a long-term debilitating disease because it may lead to eye infections and blindness.

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

At the time of designation, atopic keratoconjunctivitis affected approximately 1.5 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 76,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

#### **What treatments are available?**

At the time of submission of the application for orphan designation, several medicines were available in the EU for the treatment of atopic keratoconjunctivitis, including anti-inflammatory medicines such as corticosteroids and anti-allergic medicines such as antihistamines.

The sponsor has provided sufficient information to show that ciclosporin (eye drops, solution) might be of significant benefit for patients with atopic keratoconjunctivitis because early studies have shown that by giving this medicine locally into the eye it might have fewer side effects than existing treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

#### **How is this medicine expected to work?**

Ciclosporin is an immunosuppressant medicine (a medicine that reduces the activity of the immune system) that has been used since the early 1980s. Ciclosporin (eye drops, solution) is a new formulation of ciclosporin that is to be given as eye drops. In atopic keratoconjunctivitis, this medicine

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\*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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).

is expected to reduce inflammation of the eye by reducing the production of immune cells called 'lymphocytes'.

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

The effects of ciclosporin (eye drops, solution) have been evaluated in experimental models. At the time of submission of the application for orphan designation, a clinical trial in healthy volunteers had finished, and additional studies in patients with atopic keratoconjunctivitis were planned.

At the time of submission, ciclosporin (eye drops, solution) was not authorised anywhere in the EU for atopic keratoconjunctivitis or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 June 2009 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 Community) 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:**

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

<b>Language</b>	<b>Active ingredient</b>	<b>Indication</b>
English	Ciclosporin (eye drops, solution)	Treatment of atopic keratoconjunctivitis
Bulgarian	Циклоспорин (капки за очи, разтвор)	Лечение на atopичен кератоконюнктивит
Czech	Ciclosporin (oční kapky, roztok)	Léčba atopické keratokonjunktivitidy
Danish	Ciclosporin (øjendråber, opløsning)	Behandling af atopisk keratokonjunktivit
Dutch	Ciclosporine (oogdruppels, oplossing)	Behandeling van atopische keratoconjunctivitis
Estonian	Tsüklosporiin (silmatilgad, lahus)	Atoopilise keratokonjunktiviidi ravi
Finnish	Siklosporiini (silmätipat, liuos)	Atooppisen keratokonjunktiviitin hoito
French	Ciclosporine (collyre en solution)	Traitement des kératoconjunctivites atopiques
German	Ciclosporin (Augentropfen)	Behandlung der atopischen Keratokonjunktivitis
Greek	Κυκλοσπορίνη (οφθαλμικές σταγόνες, διάλυμα)	Θεραπεία της atopικής κερατοεπιπεφυκίτιδας
Hungarian	Cyclosporin (oldatos szemcsepp)	Atopiás keratoconjunctivitis kezelésére
Italian	Ciclosporina (collirio, soluzione)	Trattamento della cheratoconguntivite atopica
Latvian	Ciklosporīns (acu pilieni, šķīdums)	Atopiska keratokonjunktivīta ārstēšanai
Lithuanian	Ciklosporinas (akių lašai, tirpalas)	Atopinio keratokonjunktivito gydymas
Maltese	Ciclosporin (qtar għall-ghajnejn, taħlita likwida)	Kura tal-keratokonguntivite atopika
Polish	Cyklosporyna (krople do oczu, roztwór)	Leczenie atopowego zapalenia rogówki i spojówek
Portuguese	Ciclosporina (colírio, solução)	Tratamento da queratoconjuntivite atópica
Romanian	Ciclosporină (picături oftalmice, soluție)	Tratamentul keratoconjunctivitei atopice
Slovak	Cyklosporín (očná roztoková, instilácia)	Liečba atopickéj keratokonjunktivitídy
Slovenian	Ciklosporin (kapljice za oko, raztopina)	Zdravljenje atopičnega keratokonjunktivitisa
Spanish	Ciclosporina (colirio en solución)	Tratamiento de la queratoconjuntivitis atópica
Swedish	Ciklosporin (ögondroppar, lösning)	Behandling av atopisk keratokonjunktivit
Norwegian	Ciklosporin (øyedråper, oppløsning)	Behandling av atopisk keratokonjunktivitt
Icelandic	Cíklósporín (augndropar, lausn)	Meðferð við glæru- og tárubólgu vegna ofnæmis