



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Voclosporin for the treatment of non-infectious uveitis

On 6 December 2012, orphan designation (EU/3/12/1085) was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for voclosporin for the treatment of non-infectious uveitis.

#### What is non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye. The inflammation can affect one or both eyes, and may cause discomfort, pain, and blurring of vision. Non-infectious uveitis is usually caused by the body's immune system (the body's natural defences) attacking normal tissue and not by an infection.

Non-infectious uveitis is a long-term debilitating disease because it may lead to partial or complete loss of vision (blindness).

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

At the time of designation, non-infectious uveitis affected less than 4.8 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 243,000 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?

At the time of designation, several medicines were authorised in Member States of the EU for the treatment of non-infectious uveitis. Most treatments, such as corticosteroids or ciclosporin, aimed to reduce the inflammation by lowering the activity of the immune system.

The sponsor has provided sufficient information to show that voclosporin might be of significant benefit for patients with non-infectious uveitis because early studies suggest that it has fewer serious side effects than ciclosporin and may also allow lower doses of corticosteroids to be given. These

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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 506,300,000 (Eurostat 2011).



assumptions 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?**

Voclosporin is an immunosuppressant medicine known as a calcineurin inhibitor. This means that it blocks the action of an enzyme, calcineurin, important for the action of T lymphocytes, which are part of the immune system and play a role in inflammation. Voclosporin blocks the release of certain substances called lymphokines and interleukins which are needed for the normal functioning of the T lymphocytes. By reducing the action of T lymphocytes, voclosporin is expected to reduce the inflammation in non-infectious uveitis.

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

The effects of voclosporin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with voclosporin in patients with non-infectious uveitis were ongoing.

At the time of submission, voclosporin was not authorised anywhere in the EU for non-infectious uveitis. Orphan designation of voclosporin had previously been granted in the EU for chronic non-infectious uveitis, and had been granted in the United States of America for the treatment and chronic control of non-infectious posterior, intermediate and panuveitis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 07 November 2012 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 substance	indication
English	Voclosporin	Treatment of non-infectious uveitis
Bulgarian	Воклоспорин	Лечение на хроничен неинфекциозен увеит
Czech	Voclosporin	Léčba neinfekční uveitidy
Danish	Voclosporin	Behandling af non-infektios uveitis
Dutch	Voclosporine	Behandeling van niet-infectieuze uveïtis
Estonian	Voclosporiin	Mitte-infektsioosse uveidi ravi
Finnish	Voklosporiini	Ei-infektioperäisen uveitin hoito
French	Voclosporine	Traitement de l'uvéïte non infectieuse
German	Voclosporin	Behandlung der nicht-infektiösen Uveitis
Greek	Βοκλοσπορίνη	Θεραπεία της μη μολυσματικής ραγοειδίτιδας.
Hungarian	Voklosporin	Nem fertőzéses eredetű uveitis kezelése
Italian	Voclosporina	Trattamento dell'uveite non infettiva
Latvian	Voclosporīns	Ne-infekciozā uveīta ārstēšana
Lithuanian	Voklosporinas	Neinfekcinio uveito gydymas
Maltese	Voclosporin	Kura ta' uveite mhux infettiva
Polish	Voklosporyna	Leczenie nieinfekcyjnego zapalenia błony naczyniowej oka
Portuguese	Voclosporina	Tratamento da uveite não infecciosa
Romanian	Voclosporină	Tratamentul uveitei non-infectioase
Slovak	Voklosporín	Liečba neinfekčnej uveitídy
Slovenian	Voclosporin	Zdravljenje neinfekcijskega uveitis
Spanish	Voclosporina	Tratamiento de la uveítis no infecciosa
Swedish	Voklosporin	Behandling av icke-infektios uveit
Norwegian	Voklosporin	Behandling av ikke-infeksiøs uveitt
Icelandic	Vóklósporín	Meðferð á æðahjúpsbólgu án sýkingar

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