



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sirolimus for the treatment of chronic non-infectious uveitis

On 30 August 2011, orphan designation (EU/3/11/898) was granted by the European Commission to Santen Oy, Finland, for sirolimus for the treatment of chronic non-infectious uveitis.

What is chronic 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.

Chronic (long-term) 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, chronic non-infectious uveitis affected approximately 4.1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 208,000 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. The first treatment option was corticosteroids, which were used to reduce the inflammation by lowering the activity of the immune system. Other immunosuppressant agents such as ciclosporin and azathioprine were also authorised for use in chronic non-infectious uveitis.

The sponsor has provided sufficient information to show that sirolimus might be of significant benefit for patients with chronic non-infectious uveitis because it works in a different way to existing

*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).



treatments and can be administered locally, as seen in early studies. 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?

Sirolimus is an immunosuppressive agent, which has been used for several years to prevent organ rejection following a transplant. It is expected to work by blocking an enzyme called 'mammalian target of rapamycin' (mTOR). In the body, sirolimus first attaches to a protein called FKBP-12 that is found inside cells to make a 'complex' which then blocks mTOR. Since mTOR is involved in the activation and proliferation of T lymphocytes (white blood cells that play a role in inflammation), sirolimus is expected to reduce the inflammation in chronic non-infectious uveitis. Sirolimus also stops the production of growth factors and genes involved in uveitis. Sirolimus is expected to be injected into or around the eye.

What is the stage of development of this medicine?

The effects of sirolimus have been evaluated in experimental models.

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

At the time of submission, sirolimus was not authorised anywhere in the EU for chronic non-infectious uveitis or designated as an 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 10 June 2011 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 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sirolimus	Treatment of chronic non-infectious uveitis
Bulgarian	Сиροлимус	Лечение на хроничен неинфекциозен увеит
Czech	Sirolimus	Léčba chronické neinfekční uveitidy
Danish	Sirolimus	Behandling af kronisk ikke-infektiøs uveitis
Dutch	Sirolimus	Behandeling van chronische niet-infectieuze uveïtis
Estonian	Sirolimusi	Kroonilise mitteinfektsioosse uveidi ravi.
Finnish	Sirolimuusi	Kroonisen, ei-infektioperäisen uveitin hoito
French	Sirolimus	Traitement de l'uvéïte chronique non infectieuse
German	Sirolimus	Behandlung der chronischen nicht-infektiösen Uveitis
Greek	Σιρόλιμους	Θεραπεία της χρόνιας μη μολυσματικής ραγοειδίτιδας.
Hungarian	Szirolimusz	Krónikus, nem fertőzések eredetű uveitis kezelése
Italian	Sirolimus	Trattamento dell'uveite cronica non infettiva
Latvian	Sirolimus	Hroniskā neinfekciozā uveīta ārstēšana
Lithuanian	Sirolimuzas	Lėtinio neinfekcinio uveito gydymas
Maltese	Sirolimus	Kura ta' l-ujejite kronika u mhux infettiva
Polish	Syrolimus	Leczenie przewlekłego niezakaźnego zapalenia błony naczyniowej oka
Portuguese	Sirolimus	Tratamento da uveíte crónica não infecciosa.
Romanian	Sirolimus	Tratamentul uveitei non-infectioase cronice
Slovak	Sirolimus	Liečba chronickej neinfekčnej uveitídy
Slovenian	Sirolimus	Zdravljenje kroničnega neinfekcijskega uveitisa
Spanish	Sirolimus	El tratamiento de la uveitis crónica no infecciosa
Swedish	Sirolimus	Behandling av kronisk icke infektiös uveit
Norwegian	Sirolimus	Behandling av kronisk ikke-infeksiøs uveitt
Icelandic	Sírólímús	Til meðferðar á augnæðahjúpsbólgu sem ekki stafar af sýkingu

¹ At the time of designation