



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Triamcinolone acetonide for the treatment of non-infectious uveitis

On 21 May 2015, orphan designation (EU/3/15/1490) was granted by the European Commission to S-cubed Limited, United Kingdom, for triamcinolone acetonide for the treatment of non-infectious uveitis.

What is non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye, just beneath the white part 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 5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 256,000 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 were also authorised for use in non-infectious uveitis.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with non-infectious uveitis because it is given directly into the part of the eye affected by non-infectious uveitis, and early studies have shown that patients experienced fewer side

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).



effects compared with authorised corticosteroid 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?

Triamcinolone acetonide is a corticosteroid that reduces inflammation and suppresses the body's immune system. It is authorised in various formulations for many inflammatory conditions. It works by entering cells and blocking the production of vascular endothelial growth factor (VEGF) and prostaglandins, substances that are involved in swelling and inflammation. The medicine is expected to be injected into the suprachoroidal space (the portion of the eye below the white part). This will help to ensure that adequate amounts of triamcinolone acetonide are delivered to the part of the eye where the swelling in non-infectious uveitis occurs, and to avoid delivery to other parts of the eye where the medicine is not needed, thus reducing the occurrence of side effects.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for 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 16 April 2015 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	Triamcinolone acetonide	Treatment of non-infectious uveitis
Bulgarian	триамцинолон ацетонид	Лечение на хроничен неинфекциозен увеит
Croatian	Triamcinolonacetamid	Liječenje neinfektivnog uveitisa
Czech	Triamcinolon acetonidu	Léčba neinfekční uveitidy
Danish	Triamcinolonacetamid	Behandling af non-infektios uveitis
Dutch	Triamcinolonacetamide	Behandeling van niet-infectieuze uveïtis
Estonian	Triamtsinolonatsetoniid	Mitte-infektsioosse uveidi ravi
Finnish	Triamsinoloniasetonidi	Ei-infektiooperäisen uveitin hoito
French	Acétonide de triamcinolone	Traitement de l'uvéïte non infectieuse
German	Triamcinolonacetamid	Behandlung der nicht-infektiösen Uveitis
Greek	ακετονίδιο της τριαμσινολόνης	Θεραπεία της μη μολυσματικής ραγοειδίτιδας.
Hungarian	Triamcinolon-acetonid	Nem fertőzések eredetű uveitis kezelése
Italian	Triamcinolone acetonide	Trattamento dell'uveite non infettiva
Latvian	Triamcinolona acetonīds	Ne-infekciozā uveīta ārstēšana
Lithuanian	Triamcinolono acetonidas	Neinfekcinio uveito gydymas
Maltese	Triamcinolone acetonide	Kura ta' uveite mhux infettiva
Polish	Acetonid triamcynolonu	Leczenie nieinfekcyjnego zapalenia błony naczyniowej oka
Portuguese	Acetonido de triamcinolona	Tratamento da uveite não infecciosa
Romanian	Triamcinolon acetamid	Tratamentul uveitei non-infectioase
Slovak	Triamcinolónacetamide	Liečba neinfekčnej uveitídy
Slovenian	Triamcinolonacetamid	Zdravljenje neinfekcijskega uveitis
Spanish	Acetónido de triamcinolona	Tratamiento de la uveitis no infecciosa
Swedish	Triamcinolonacetamid	Behandling av icke-infektios uveit
Norwegian	Triamcinolonacetamid	Behandling av ikke-infeksiøs uveitt
Icelandic	Tríamcínólón acetóníð	Meðferð á æðahjúpsbólgu án sýkingar

¹ At the time of designation