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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dexamethasone (intravitreal implant) for the treatment of non-infectious uveitis affecting the posterior segment of the eye

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in August 2010 on request of the sponsor.

On 4 August 2010, orphan designation (EU/3/10/763) was granted by the European Commission to Allergan Pharmaceuticals Ireland, Ireland, for dexamethasone (intravitreal implant) for the treatment of non-infectious uveitis affecting the posterior segment (the back) of the eye.

What is non-infectious uveitis affecting the posterior segment of the eye?

Uveitis is inflammation of the uvea, the middle layer of the eye. The inflammation can be localised only to the back of one or both eyes, and may cause discomfort, pain, and blurring of the 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 affecting the posterior segment of the eye is a long-term debilitating disease because it may lead to partial or complete loss of vision.

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

At the time of designation, non-infectious uveitis affecting the posterior segment of the eye affected between 0.3 and 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 15,000 and 51,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 506,500,000 (Eurostat 2010).

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 aimed to reduce the inflammation by lowering the activity of the immune system. Dexamethasone eye drops and oral solution were authorised for non-infectious uveitis and posterior uveitis, respectively.

The sponsor has provided sufficient information to show that dexamethasone (intravitreal implant) might be of significant benefit for patients with non-infectious uveitis affecting the posterior segment of the eye because results from clinical studies indicate that it might improve the outcome of patients with this condition. In addition, this medicine might contribute to the care of patients because of the way it is expected to carry the dexamethasone to the back of the eye. These 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?

Dexamethasone belongs to a group of anti-inflammatory medicines known as corticosteroids. It helps reduce inflammation by attaching to receptors of immune cells and moderating the activity of the immune system.

Dexamethasone (intravitreal implant) is an implant to be injected into the eye. The implant is made of a biodegradable material which dissolves over several months while slowly releasing dexamethasone inside the eye.

What is the stage of development of this medicine?

The effects of dexamethasone (intravitreal implant) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with dexamethasone (intravitreal implant) in patients with non-infectious uveitis affecting the posterior segment of the eye were ongoing.

At the time of submission, dexamethasone (intravitreal implant) was authorised in the United States of America for macular oedema (swelling of the macula, the central part of the retina).

At the time of submission, dexamethasone (intravitreal implant) was not authorised anywhere in the EU for non-infectious uveitis affecting the posterior segment of the eye. Orphan designation of this medicine had been granted in the United States of America for non-infectious ocular inflammation of the posterior segment in patients with intermediate, posterior and panuveitis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 May 2010 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	Dexamethasone (intravitreal implant)	Treatment of non-infectious uveitis affecting the posterior segment of the eye
Bulgarian	Дексаметазон (имплантант за стъкловидно тяло)	Лечение на неинфекциозен увеит на задния очен сегмент
Czech	Dexametazon (intravitreální implantát)	Léčba neinfekční uveitidy postihující zadní segment oka
Danish	Dexamethason (intravitreal implantat)	Behandling af ikke-infektøs uveitis, som påvirker øjets bageste segment
Dutch	Dexamethasone (intravitreaal implantaat)	De behandeling van niet-besmettelijke uveitis in het achterste deel van het oog
Estonian	Deksametasoon (intravitreaalne implantaat)	Silma tagumist segmenti hõlmava mitteinfektsioosse uveiidi ravi
Finnish	Deksametasoni (intravitreaalinen implantti)	Silmän takasegmentin käsittävän, infektoitumattoman uveitin hoitoon
French	Dexaméthasone (implant intravitréen)	Le traitement des uvéites non-infectieuses affectant le segment postérieur de l'œil
German	Dexamethason (intravitreales Implantat)	Behandlung von nichtinfektiöser Uveitis des hinteren Augensegments.
Greek	Δεξαμεθαζόνη (ενδοϋαλοειδικό μόσχευμα)	Θεραπευτική αγωγή για τη μη λοιμώδη ραγοειδίτιδα που επηρεάζει το οπίσθιο τμήμα του οφθαλμού
Hungarian	Dexametazon (intravitreális implantátum)	A szem hátsó szegmensét érintő, nem fertőzőes eredetű uveitis kezelése
Italian	Desametasone (impianto intravitreale)	Trattamento dell'uveite non infettiva con coinvolgimento del segmento posteriore dell'occhio
Latvian	Deksametazons (intravitreāls implantāts)	Neinfekcioza mugurējā uveīta ārstēšana
Lithuanian	Deksametazonas (vidinis stiklakūnio implantas)	Neinfekcinio uveito gydymas, apimantis užpakalinį akies segmentą
Maltese	Dexamethasone (impjant intravitreali)	Kura ta' uveite mhux infettiva li tolqot il-parti ta' wara tal-għajn
Polish	Deksametazon (implant do ciała szklistego)	Leczenie nieinfekcyjnego zapalenia błony naczyniowej w obrębie tylnego odcinka oka
Portuguese	Dexametasona (implante intravítreo)	O tratamento de uma uveíte não infecciosa que afecta o segmento posterior do olho
Romanian	Dexametazonă (implant intravitros)	Tratamentul uveitei non-infectioase cu afectarea segmentului posterior al ochiului
Slovak	Dexametazón (intravitreálny implantát)	Liečba neinfekčnej uveitídy zasahujúcej zadnú časť oka
Slovenian	Deksametazon (intravitrealni implantat)	Zdravljenje nenalezljivega uveitisa, ki zajema posteriorni del očesa

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Dexametasona (implante intravítreo)	El tratamiento de uveítis no infecciosas que afectan al segmento posterior del ojo
Swedish	Dexametason (intravitrealt implantat)	Behandling av icke-infektiös uveit som berör ögats bakre segment
Norwegian	Deksametason (intravitrealt implantat)	Behandling av ikke-smittsom uveitt, som påvirker bakre del av øyet
Icelandic	Dexametasón (vefjalyf til notkunar í glerhlaup augans)	Meðferð við æðahjúpsbólgu sem ekki er rakin til sýkingar og leggst á aftari hluta augans