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EMA/COMP/242323/2016  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Fluocinolone acetonide for the treatment of non-infectious uveitis

On 28 April 2016, orphan designation (EU/3/16/1647) was granted by the European Commission to Campharm Ltd, United Kingdom, for fluocinolone 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 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 was equivalent to a total of fewer than 247,000 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. These included Ozurdex (dexamethasone) given by injection into the eye. Other immunosuppressant medicines such as ciclosporin were also authorised for use in non-infectious uveitis.

The sponsor has provided sufficient information to show that fluocinolone acetonide might be of significant benefit for patients with non-infectious uveitis because the medicine is formulated to

\*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 513,700,000 (Eurostat 2016).



provide a prolonged effect over 3 years after injection into the eye, which is much longer 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?

Fluocinolone 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 blocking the production of substances that are involved in swelling and inflammation. This medicine is contained in a tiny implant that is injected into the inflamed part of the eye using a special needle. Following injection, the implant releases the corticosteroid over a 3-year period. This helps to ensure that fluocinolone acetonide is delivered long-term to the part of the eye where the inflammation occurs in non-infectious uveitis, and helps avoid transfer to other parts of the body where the medicine is not needed, thus reducing side effects.

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

The effects of fluocinolone acetonide have been evaluated in experimental models.

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

At the time of submission, fluocinolone acetonide was not authorised anywhere in the EU for non-infectious uveitis or designated as an orphan medicinal product elsewhere for this condition. A similar product was available in some EU member states under the trade name Iluvien for the treatment of diabetic macular oedema.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 23 March 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 ingredient              | Indication   |
|------------|--------------------------------|--|
| English    | Fluocinolone acetonide         | Treatment of non-infectious uveitis                      |
| Bulgarian  | Флуоцинолон ацетонид           | Лечение на хроничен неинфекциозен увеит                  |
| Croatian   | Fluocinolon acetonid           | Liječenje neinfektivnog uveitisa                         |
| Czech      | Fluocinolon acetonid           | Léčba neinfekční uveitidy                                |
| Danish     | Fluocinolonacetonid            | Behandling af non-infektiøs uveitis                      |
| Dutch      | Fluocinolonacetonide           | Behandeling van niet-infectieuze uveïtis                 |
| Estonian   | Fluotsinoloonatsetoniid        | Mitte-infektsioosse uveidi ravi                          |
| Finnish    | Fluokinoloniasetonidi          | Ei-infektioperäisen uveiitin hoito                       |
| French     | Acétonide de fluocinolone      | Traitemennt de l'uvéite non infectieuse                  |
| German     | Fluocinolonacetonid            | Behandlung der nicht-infektiösen Uveitis                 |
| Greek      | Ακετονίδιο της φλουσοκινολόνης | Θεραπεία της μη μολυσματικής ραγοειδίτιδας.              |
| Hungarian  | Fluocinolon acetonid           | Nem fertőzéses eredetű uveitis kezelése                  |
| Italian    | Acetonide di fluocinolone      | Trattamento dell'uveite non infettiva                    |
| Latvian    | Fluocinolona acetonīds         | Ne-infekcīzā uveīta ārstēšana                            |
| Lithuanian | Fluocinolono acetonidas        | Neinfekcinio uveito gydymas                              |
| Maltese    | Fluocinolone acetonide         | Kura ta' uveite mhux infettiva                           |
| Polish     | Acetonid fluocinolonu          | Leczenie nieinfekcyjnego zapalenia błony naczyniowej oka |
| Portuguese | Acetonido de fluocinolona      | Tratamento da uveíte não infecciosa                      |
| Romanian   | Fluocinolon acetonid           | Tratamentul uveitei non-infecțioase                      |
| Slovak     | Fluocinolón acetonid           | Liečba neinfekčnej uveitídy                              |
| Slovenian  | Fluocinolon acetonid           | Zdravljenje neinfekcijskega uveitis                      |
| Spanish    | Acetónido de fluocinolona      | Tratamiento de la uveítis no infecciosa                  |
| Swedish    | Fluokinolonacetonid            | Behandling av icke-infektiös uveit                       |
| Norwegian  | Fluocinolonacetonid            | Behandling av ikke-infeksiøs uveitt                      |
| Icelandic  | Flúócinólón acetóníð           | Meðferð á æðahjúpsbólgu án sýkingar                      |

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