



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Methotrexate for the treatment of retinal detachment

On 4 June 2020, orphan designation EU/3/20/2276 was granted by the European Commission to Helio Vision Germany GmbH, Germany, for methotrexate for the treatment of retinal detachment.

What is retinal detachment?

Retinal detachment is the separation of the thin, light-sensitive tissue at the back of the eye from the layer of blood vessels that supply nutrients and oxygen.

The condition affects vision, and the patient may see specks floating across the eye or sense flashing light or a curtain-like dark shadow. Vision may become blurred and the patient may start to lose vision at the edges.

Some patients may have proliferative vitreoretinopathy, a complication that can occur after surgery to repair the detachment. The complication can lead to abnormalities in the layers around the retina as well as scarring and build-up of fluids which can cause the retina to pull away again.

Retinal detachment is disabling in the long term because of extensive loss of vision.

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

At the time of designation, retinal detachment affected approximately 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 114,000 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?

No medicines to treat retinal detachment were authorised in the EU at the time of application. Retinal detachment was generally treated by surgery, sometimes with injection of gas or oil into the eye to push the retina back into place or the use of lasers or freezing of the affected area.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



How is this medicine expected to work?

Methotrexate is aimed at preventing proliferative vitreoretinopathy, a complication of retinal detachment. In patients with this complication, the cells around the detached retina grow uncontrollably. Methotrexate blocks the action of a protein called dihydrofolate reductase, which is needed for cells to grow and multiply. It is expected that injecting methotrexate into the eye will reduce the abnormal growth of cells and so slow down proliferative vitreoretinopathy and reduce the loss of vision.

Methotrexate has been used for many years in the EU in the treatment of cancer and other long-term diseases such as rheumatoid arthritis where it blocks the growth of cells and tissues by interfering with the production of new DNA.

What is the stage of development of this medicine?

The effects of methotrexate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with methotrexate in patients with retinal detachment were ongoing.

At the time of submission, methotrexate was not authorised anywhere in the EU for the treatment of retinal detachment. Orphan designation of methotrexate had been granted in the United States for the prevention of proliferative vitreoretinopathy.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 23 April 2020, 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:

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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	Methotrexate	Treatment of retinal detachment
Bulgarian	Метотрексат	Лечение на отлепване на ретината
Croatian	Metotreksat	Liječenje odljepljenja mrežnice
Czech	Methotrexate	Léčba odchlípení sítnice
Danish	Methotrexate	Behandling af nethindeløsning
Dutch	Methotrexate	Behandeling van netvliesloslating
Estonian	Metotreksaat	Reetina irdumise ravi
Finnish	Metotreksaatti	Verkkokalvoirtauman hoito
French	Méthotrexate	Traitement du décollement de la rétine
German	Methotrexat	Behandlung von Netzhautablösung
Greek	Μεθοτρεξάτη	Θεραπεία της αποκόλλησης του αμφιβληστροειδούς
Hungarian	Methotrexate	Retinaleválás kezelése
Italian	Metotrexato	Trattamento del distacco di retina
Latvian	Metotreksāts	Tīklenes atslāņošanās ārstēšana
Lithuanian	Metotreksatas	Tinklainės atšoka
Maltese	Methotrexate	Trattament ta' distakk tar-retina
Polish	Metotreksat	Leczenie oklejenia siatkówki
Portuguese	Metotrexato	Behandeling van netvliesloslating
Romanian	Metotrexat	Tratamentul detașării retinei
Slovak	Metotrexát	Liečba oddelenia sietnice
Slovenian	Methotreksat	Zdravljenje odstopa mrežnice
Spanish	Metotrexato	Tratamiento del desprendimiento de la retina
Swedish	Methotrexat	Behandling av näthinneavlossning
Norwegian	Metotreksat	Behandling av netthinneløsning
Icelandic	Methotrexat	Meðferð við sjónulosi

¹ At the time of designation