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Public summary of opinion on orphan designation

Retinol for the prevention of retinopathy of prematurity

On 17 July 2017, orphan designation (EU/3/17/1895) was granted by the European Commission to orphanix GmbH, Austria, for retinol for the prevention of retinopathy of prematurity.

What is retinopathy of prematurity?

Retinopathy of prematurity is an eye condition that occurs in babies who are born prematurely. A baby's blood supply to the eye develops mainly in the last few weeks of pregnancy. When a baby is born too early, blood vessels in the eye may not develop normally. Instead, abnormal and fragile blood vessels may develop, resulting in damage to the retina, the area at the back of the eye which detects light and is essential for normal vision.

Retinopathy of prematurity is debilitating in the long term because it can reduce sight and may cause blindness.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of retinopathy of prematurity was estimated to be approximately 1.4 people in 10,000 in the European Union (EU). This was equivalent to a total of around 72,000 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 methods of prevention are available?

At the time of the designation, no satisfactory method was authorised in the EU for the prevention of retinopathy of prematurity. Treatment once the condition began to develop consisted of laser or cryotherapy (treatment of the affected area using low temperature) to close the abnormal blood vessels that damage the retina.

*Disclaimer: For the purpose of the designation, the number of patients at risk of developing 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 515,700,000 (Eurostat 2017).

How is this medicine expected to work?

Retinol is a form of vitamin A. It prevents damage to cells in the eye and is involved in maintaining the health of the retina and other parts of the eye. Retinol also controls a substance called 'vascular endothelial growth factor' which helps to develop healthy blood vessels. It is expected that giving retinol by injection will reduce damage to the retina and maintain eye health in babies at risk of retinopathy of prematurity.

What is the stage of development of this medicine?

The effects of retinol have been evaluated in experimental models. As retinol is a well-known substance, the sponsor provided data in patients from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in babies at risk of retinopathy of prematurity were ongoing.

At the time of submission, retinol was not authorised anywhere in the EU for prevention of retinopathy of prematurity 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 15 June 2017 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, 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Retinol	Prevention of retinopathy of prematurity
Bulgarian	Ретинол	Превенция на ретинопатия при недоносени
Croatian	Retinol	Za prevenciju retinopatije nedonoščadi
Czech	Retinol	Prevence retinopatie nedonošenců
Danish	Retinol	Forebyggelse af præmatur retinopati
Dutch	Retinol	Preventie van retinopathie van de prematuur
Estonian	Retinool	Enneaegsete retinopaatia ennetamiseks
Finnish	Retinoli	Keskosen retinopatian esto
French	Rétinol	Prévention de la rétinopathie du prématuré
German	Retinol	Prävention der Frühgeborenen-Retinopathie
Greek	Ρετινόλη	Πρόληψη αμφιβληστροειδοπάθειας της προωρότητας
Hungarian	Retinol	Koraszülöttkori retinopátia megelőzése
Italian	Retinolo	Prevenzione della retinopatia della prematurita'
Latvian	Retinols	Priekšlaikus dzimušu bērnu retinopātijas profilakse
Lithuanian	Retinolis	Neišnešiotų naujagimių retinopatijos prevencija
Maltese	Retinol	Prevenzjoni ta' retinopatija ta' prematurità
Polish	Retinol	Zapobieganie retinopatii wcześniaczej
Portuguese	Retinol	Prevenção da retinopatia da prematuridade
Romanian	Retinol	Prevenirea retinopatiei de prematuritate
Slovak	Retinol	Prevenca retinopatie u predčasne narodených
Slovenian	Retinol	Preprečevanje retinopatije nedonošenčkov
Spanish	Retinol	Prevención de la retinopatía de los prematuros
Swedish	Retinol	Förebyggande av prematuritetsretinopati
Norwegian	Retinol	Forebygging av prematuritetsretinopati
Icelandic	Retínól	Forvörn gegn fyrirbura sjónukvilla

¹ At the time of designation