



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Propranolol hydrochloride for the treatment of retinopathy of prematurity

On 17 October 2019, orphan designation EU/3/19/2217 was granted by the European Commission to Recordati Rare Diseases, France, for propranolol hydrochloride for the treatment 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 affected by the condition?

At the time of designation, retinopathy of prematurity affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 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 the designation, no satisfactory method of treatment was authorised in the EU for 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 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 518,400,000 (Eurostat 2019).



How is this medicine expected to work?

Propranolol hydrochloride blocks a target called beta-adrenoreceptor which is involved in the production of VEGF, a protein involved in the growth of blood vessels. By blocking the action of this receptor, the medicine is expected to reduce the formation of new blood vessels which occurs in patients with retinopathy of prematurity. This should reduce the symptoms of the disease. The medicine is expected to be available as eye drops.

What is the stage of development of this medicine?

The effects of propranolol hydrochloride have been evaluated in experimental models.

Propranolol hydrochloride is authorised in some EU countries to treat various conditions, and is taken by mouth or injected into a vein.

At the time of submission of the application for orphan designation, clinical trials with propranolol hydrochloride in patients with retinopathy of prematurity were ongoing.

At the time of submission, propranolol hydrochloride was not authorised anywhere in the EU for the treatment of retinopathy of prematurity or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 12 September 2019, 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	Propranolol hydrochloride	Treatment of retinopathy of prematurity
Bulgarian	Пропранолол хидрохлорид	Лечение на ретинопатия на недоносени деца
Croatian	Propranolol hidroklorid	Liječenje prematurne retinopatije
Czech	Propranolol hydrochlorid	Léčba retinopatie nedonošených dětí
Danish	Propranololhydrochlorid	Behandling af retinopati hos præmature børn
Dutch	Propranolol hydrochloride	Behandeling van retinopathie van de prematuur
Estonian	Propranolool hüdrokloriid	Enneaegsete retinopaatia ravi
Finnish	Propranololi hydrokloridi	Keskosen retinopatian hoito
French	Propranolol chlorhydrate	Traitement de la rétinopathie du prématuré
German	Propranolol Hydrochlorid	Behandlung der Netzhauterkrankung bei Frühgeborenen
Greek	Προπρανολόλη υδροχλωρική	Θεραπεία αμφιβληστροειδοπάθειας της προωρότητας
Hungarian	Propranolol hidroklorid	A koraszülött retinopátiájának kezelésére
Italian	Propranololo cloridrato	Tattamento della retinopatia del prematuro
Latvian	Propranolola hidrohlorīds	Priekšlaikus dzimušo retinopātijas ārstēšana
Lithuanian	Propranololio hidrochloridas	Neišnešiotų naujagimių retinopatijai retinopatijos gydytigydyimas
Maltese	Propranolol kloridrat	Ttrattament ta' Retinopatija tal-Prematurità
Polish	Propranolol chlorowodorek	Leczenie retinopatii wcześniaków
Portugues e	Cloridrato propranolol	Tratamento da retinopatia do prematuro
Romanian	Propranolol clorhidrat	Tratamentul retinopatiei de prematuritate
Slovak	Propranolol hydrochlorid	Liečba retinopatie nedonosených detí
Slovenian	Propranololijev klorid	Zdravljenje retinopatije nedonošenčkov
Spanish	Cloridrato de propranolol	Tratamiento de la retinopatía de los prematuros
Swedish	Propranolol hydroklorid	Behandling av prematuritetsretinopati
Norwegian	Propranololhydroklorid	Behandling av prematuritetsretinopati
Icelandic	Própranólól hýdróklóríð	Meðferð við sjónukvilla fyrirbura

¹ At the time of designation