European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of mecasermin rinfabate for the prevention of retinopathy of prematurity in neonates of less than 32 weeks of gestational age

On 28 August 2006, orphan designation (EU/3/06/399) was granted by the European Commission to ROP Pharma AB, Sweden, for mecasermin rinfabate for the prevention of retinopathy of prematurity in neonates of less than 32 weeks of gestational age.
The sponsor changed name to Premacure AB in September 2007.

What is the retinopathy of prematurity?
Children that are born preterm are deprived of their natural environment in the womb, and they lack important factors normally provided to the unborn child, such as proteins, cellular growth factors and cytokines. The last weeks of a full-term pregnancy are also important for the growth of the eye, in particular for the formation of blood vessels supplying blood to the retina (the area at the back of the eye that receives light and sends pictures of what the eye sees to the brain). In some premature infants, the normal growth of the retinal vessels stops, and abnormal new vessels begin to grow. Therefore, the oxygen supply to the retina is limited. The formation of abnormal vessels is accompanied by the production of scar tissue. In some cases this can result in visual impairment and in extreme cases in blindness.

What is the estimated number of patients at risk of developing the condition?
At the time of designation the population at risk of developing retinopathy of prematurity in neonates of less than 32 weeks of gestational age was approximately 1.2 in 10,000 people in the European Union (EU) [*]. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 55,000 people.

What methods of prevention are available?
At the time of the submission of orphan drug designation application, there were no medicinal products approved for the prevention of condition in the Community. The treatment consisted of laser or cryotherapy (local treatment with "ice") to close the abnormal new vessels and to limit the scar tissue.

How is this medicine expected to work?
One factor that stimulates cell growth and helps cells to develop special features for specific functions (specialization) is insulin-like growth factor 1 (IGF-1). A lack of it may lead to abnormal vessel growth in retina that is typical for retinopathy of prematurity. IGFBP-3 is a protein that binds to IGF-I

[*] Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.europa.eu http://www.emea.europa.eu

and regulates the availability and activity of IGF-1. Mecasermin rinfabate mimics the effects of this natural protein complex (IGF-1/IGFBP-3) in the bloodstream and is able to stay in the body for a longer period. Therefore, the product is expected to promote the normal physiological development of the eye in premature neonates.

What is the stage of development of this medicine?
The effects of the medicinal product were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with condition were initiated.

Mecasermin rinfabate was not authorised anywhere worldwide for prevention of retinopathy of prematurity in neonates of less than 32 weeks of gestational age or designated as orphan medicinal product for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 24 July 2006 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:
Sponsor's contact details:
Premacure AB
Uppsala Science Park
SE-751 83 Uppsala
Sweden
Telephone: +46 70 650 28 52
Telefax: +46 18 572 350
E-mail: info@premacure.com

Patients' associations contact points:

Pro Retina Deutschland e.V.
Vaalser Straße 108
52074 Aachen
Germany
Telephone: +49 241 87 00 18
Telefax: +49 241 87 39 61
E-mail: Pro-Retina@t-online.de

BRPS - British Retinitis Pigmentosa Society
PO Box 350
Buckingham
MK18 1GZ
United Kingdom
Telephone: +44 1280 82 13 34
Telefax: +44 1280 81 59 00
E-mail: info@brps.org.uk

IRRP - Association Information Recherche sur la Rétinite Pigmentaire
53 Rue de la République
30160 Besseges
France
Telephone: +33 4 66 25 13 54
Telefax: +33 4 66 25 13 54
E-mail: contact@irrp.asso.fr

Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Mecasermin rinfabate	Prevention of retinopathy of prematurity in neonates of less than 32 weeks of gestational age
Czech	Mecasermin rinfabát	Prevence retinopatie nedonošených s věkem nižším než 32 týdnů gestačního věku
Danish	Mecasermin rinfabat	Forebyggelse af retinopati hos præmature børn født før 32. gestationsuge
Dutch	Mecasermin-rinfabaat	Preventie van retinopathie van de prematuur bij pasgeborenen met een draagtijd van minder dan 32 weken
Estonian	Mekasermiinrinfabaat	Enneaegsete retinopaatia ennetamiseks alla 32. rasedusnädalat sündinud vastsündinutel
Finnish	Mekasermiinirinfabaatti	Keskosen, jonka sikiöikä on alle 32 viikkoa, retinopatian esto
French	Mécasermine rinfabate	Prévention de la rétinopathie du prématuré chez les nouveau-nés de moins de 32 semaines de gestation
German	Mecasermin-Rinfabat	Prävention der Retinopathiebei Frühgeborenen mit einem Gestationsalter von unter 32 Wochen
Greek	Μηκασερμίνη rinfabate	Πρόληψη αμφιβληστροειδοπάθειας των προώρων σε βρέφη ηλικίας κύησης μικρότερης των 32 εβδομάδων
Hungarian	Mecasermin rinfabát	Retinopathia megelőzése 32 hétnél rövidebb gesztációs időt követően született koraszülötteknél
Italian	Mecasermina rinfabato	Prevenzione della retinopatia del prematuro in neonati di età gestazionale inferiore alle 32 settimane
Latvian	Mekaserminrinfabāts	Retinopātijas profilakse jaundzimušajiem, kas dzimuši pirms 32. grūtniecības nedēļas
Lithuanian	Mekasermino rinfabatas	Neišnešiotų naujagimių, gimusių iki 32 nėštumo savaitės, retinopatijos profilaktika
Polish	Rinfabat mekaserminy	Zapobieganie retinopatii wcześniaczej u noworodków urodzonych przed 32 tygodniem wieku ciążowego
Portuguese	Mecasermina rinfabato	Precenção da retinopatia do prematuro em recém-nascidos com menos de 32 semanas de idade gestacional
Slovak	Mekasermíniumrinfabát	Prevencia retinopatie u predčasne narodených detí mladších ako 32 týždňov gestačného veku
Slovenian	Mekaserminrinfabat	Preprečevanje retinopatije nedonošenčkov pri nedonošenčkih z manj kot 32 tedni gestacijske starosti
Spanish	Mecasermina rinfabato	Prevención de la retinopatía de los prematuros en neonatos prematuros con edad gestacional inferior a 32 semanas
Swedish	Mekasermin rinfabate	Profylax av prematuritetsretinopati hos prematurt nyfödda barn under 32 veckors gestationsålder
Norwegian	Mekaserminrinfabate	Forebygging av prematuritetsretinopati hos nyfødte med gestasjonsalder mindre enn 32 uker
Icelandic	Mecasermín rínfabat	Forvörn gegn sjónukvilla hjá fyrirburum hjá fyrirburum eftir minna en 32 vikna meðgöngu