The European Agency for the Evaluation of Medicinal Products
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 antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT) for the treatment of retinopathy of prematurity

On 2 October 2003, orphan designation (EU/3/03/160) was granted by the European Commission to Gene Signal SAS, France, for antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT) for the treatment of retinopathy of prematurity.

What is retinopathy of prematurity?
The last 12 weeks of a full-term delivery, from the 28th to 40th week of pregnancy, are particularly important for the growth of the eye, in particular for the formation of blood vessels, which supply 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. The formation of these abnormal vessels is accompanied by the production of scar tissue. In some cases this can result in visual impairment, in extreme cases in blindness.

What are the methods of treatment available?
There are no medicinal products approved for the condition in the Community. However, in the past two decades there has been a very significant improvement in the treatment of retinopathy of prematurity, in particular with the advances of laser and cryotherapy (local treatment with "ice").

What is the estimated number of patients affected by the condition?
According to the information provided by the sponsor, retinopathy of prematurity was considered to affect less than 57,000 newborns in the European Union.

How is this medicinal product expected to act?
The medicinal product is a very short fragment of genetic code (DNA), which specifically blocks the production of a protein directly involved in the vessel growth stimulation. Thus once administered on the eye the medicinal product is intended to locally prevent the growth of new blood vessels and thus prevent visual impairment

What is the stage of development of this medicinal product?
The evaluation of the effects of this antisense oligonucleotide in experimental models is ongoing.
At the time of submission of the application for orphan designation, no clinical trials in patients with retinopathy of prematurity were initiated.

The medicinal product was not marketed anywhere worldwide for retinopathy of prematurity or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 30 July 2003 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for 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 will be necessary before this product can be granted a marketing authorisation.

For more information:

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Patients' associations contact points:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Antisense Oligonucleotide (TATCCGGAGGGCTCGC CATGCTGCT)	Treatment of retinopathy of prematurity
Danish	Antisense Oligonucleotid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandling af retinopati hos præmature børn
Dutch	Antisense oligonucleotide (TATCCGGAGGGCTCGC CATGCTGCT)	Behandeling van retinopathie van de prematuur
Finnish	Antisense Oligonukleotidi (TATCCGGAGGGCTCGC CATGCTGCT)	Keskosen retinopatian hoito
French	Oligonucléotide antisens (TATCCGGAGGGCTCGC CATGCTGCT)	Traitement de la rétinopathie du prématuré
German	Antisense Oligonucleotid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandlung der Netzhauterkrankung bei Frühgeborenen
Greek	αντιπληροφοριακό ολιγονουκλεοτίδιο (TATCCGGAGGGCTCGC CATGCTGCT)	Θεραπεία αμφιβληστροειδοπάθειας σε πρόωρα νεογνά
Italian	Oligonucletide antisenso (TATCCGGAGGGCTCGC CATGCTGCT)	Trattamento della retinopatia del prematuro
Portuguese	Oligonucleótido Antisenso (TATCCGGAGGGCTCGC CATGCTGCT)	Tratamento da retinopatia do prematuro
Spanish	Oligonuleótido antisentido (TATCCGGAGGGCTCGC CATGCTGCT)	Tratamiento de la retinopatía de los prematuros
Swedish	Antisens oligonukletid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandling av prematuritetsretinopati