



Document Date: London, 26 April 2004
EMA/COMP/1421/03

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

**PUBLIC SUMMARY OF
POSITIVE OPINION FOR ORPHAN DESIGNATION
OF
antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT)
for treatment of neovascular glaucoma**

On 2 October 2003, orphan designation (EU/3/03/161) was granted by the European Commission to Gene Signal SAS, France, for antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT) for the treatment of neovascular glaucoma.

What is neovascular glaucoma?

Glaucoma refers to certain eye diseases that affect the nerve of the eye and can cause vision loss. Most of these diseases typically produce gradual increase of the pressure in the eye leading to the injury of the nerve. In neovascular glaucoma the normal drainage canals within the eye are physically blocked by growth of new vessels. Diabetes, too high blood pressure, an excess of cholesterol in the blood and the presence of elements obstructing some vessels, represent significant risk factors for the development of neovascular glaucoma.

The condition is chronically debilitating, in particular due to uncontrolled high pressure inside the eye and loss of vision.

What are the methods of treatment available?

At the time of submission of the application for orphan drug designation, the treatment of neovascular glaucoma consisted of various surgical procedures and authorised medicines.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that their medicinal product might be of potential significant benefit for the treatment of neovascular glaucoma. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status

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

According to the information provided by the sponsor, neovascular glaucoma was considered to affect about 75,000 to 113,000 persons 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.

What is the stage of development of this medicinal product?

The effects of antisense oligonucleotide (TATCCGGAGGGCTCGCCATGCTGCT) were evaluated in experimental models.

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

The medicinal product was not marketed anywhere worldwide for neovascular glaucoma or designated as orphan medicinal product elsewhere 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 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:

Sponsor's contact details:

Gene Signal SAS

4 Rue Pierre Fontaine

F-91000 Evry

France

Telephone: (33-675) 24 49 99

Telefax: (33-160) 878 999

E-mail: salman.almahmood@wanadoo.fr

Patients' associations contact points:

International Glaucoma Association

108C Warner Road

London

SE5 9HQ

United Kingdom

Telephone : (44-20) 77 37 32 65

Telefax: (44-20) 73 46 59 29

Email: info@iga.org.uk

*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 neovascular glaucoma
Danish	Antisense Oligonucleotid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandling af neovaskulært glaukom
Dutch	Antisense oligonucleotide (TATCCGGAGGGCTCGC CATGCTGCT)	Behandeling van neovasculair glaucom
Finnish	Antisense Oligonukleotidi (TATCCGGAGGGCTCGC CATGCTGCT)	Neovaskulaarisen glaukooman hoito
French	Oligonucléotide antisens (TATCCGGAGGGCTCGC CATGCTGCT)	Traitement du glaucome néovasculaire
German	Antisense Oligonucleotid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandlung von neovaskulärem Glaukom
Greek	αντιπληροφοριακό ολιγονουκλεοτίδιο (TATCCGGAGGGCTCGC CATGCTGCT)	Θεραπεία του νεοαγγειακού γλαυκώματος
Italian	Oligonucleotide antisenso (TATCCGGAGGGCTCGC CATGCTGCT)	Trattamento del glaucoma neovascolare
Portuguese	Oligonucleótido Antisenso (TATCCGGAGGGCTCGC CATGCTGCT)	Tratamento do glaucoma neovascular
Spanish	Oligonucleótido antisentido (TATCCGGAGGGCTCGC CATGCTGCT)	Tratamiento del glaucoma neovascular
Swedish	Antisens oligonukletid (TATCCGGAGGGCTCGC CATGCTGCT)	Behandling av neovaskulärt glaukom