



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

oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt for the treatment of glioma

On 28 October 2005, orphan designation (EU/3/05/327) was granted by the European Commission to Oligovax, France, for oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt for the treatment of glioma.

What is glioma?

Tumours that begin in brain tissue are known as primary brain tumours. Primary brain tumours are classified by the type of tissue from which they originate, the most common being gliomas, which begin in the glial (supportive) tissue.

Gliomas represent a potentially debilitating and life-threatening condition. Patients affected by gliomas can suffer from severe symptoms of the nervous system, depending on where in the brain the tumour develops.

What are the methods of treatment available?

Treatment for gliomas depends on a number of factors and encompasses several methods such as surgery, radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) or chemotherapy (using drugs to kill cancer cells), as well as some symptomatic treatments. Symptomatic treatments include certain steroid hormones (corticosteroids) to control the effects of raised pressure within the skull, and medication to help control seizures, as required. Methods of treatment of glioma were authorised in the Community at the time of submission of the application for orphan designation. Oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt might be of potential significant benefit for the treatment of gliomas because it might act in a different way and thereby it might improve the long-term outcome of the patients. 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, glioma was considered to affect about 46,000 persons in the European Union.

How is this medicinal product expected to act?

The proposed oligonucleotide phosphorothioate is a synthetic compound designed to mimic certain parts of the fundamental genetic material of the bacterial cell (bacterial DNA or CpG-oligodeoxynucleotide). Once administered directly into the brain tumour, the product is expected to stimulate the body's defence system (immune system) in the tumour area. This would then start-up a cascade of molecular reactions against the glioma and that could eventually kill the glioma cells.

What is the stage of development of this medicinal product?

The effects of oligonucleotide phosphorothioate (TAAACGTTATAACGTTATGACGTCAT), sodium salt were evaluated in experimental models.

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At the time of submission of the application for orphan designation, clinical trials in patients with glioma were ongoing.

Oligonucleotide phosphorothioate (TAAACGTTATAACGTT ATGACGTCAT), sodium salt was not authorised anywhere worldwide for glioma 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 9 September 2005 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:

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*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 Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Patients' associations contact points:

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

Language	Active Ingredient	Indication
English	Oligonucleotide phosphorothioate (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sodium salt	Treatment of glioma
Czech	Fosforothioát oligonukleotid (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sodná sůl	Léčba gliomů
Danish	Phosphorothioatoligonukleotid (<i>TAAACGTTATAACGTTATGACGTCAT</i>), natrium salt	Behandling af gliom
Dutch	Fosforothioaatoligonucleotide (<i>TAAACGTTATAACGTTATGACGTCAT</i>) natriumzout	Behandeling van glioma
Estonian	Fosforotioaatoligonukleotiidi (<i>TAAACGTTATAACGTTATGACGTCAT</i>), naatriumisool	Glioomi ravi
Finnish	Phosphorothioaatin oligonukleotidi (<i>TAAACGTTATAACGTTATGACGTCAT</i>), natriumsuola	Gliooman hoito
French	Oligonucléotide phosphorothioate (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sel sodique	Traitement des gliomes
German	Oligonukleotid phosphorthioat (<i>TAAACGTTATAACGTTATGACGTCAT</i>), Natriumsalz	Behandlung von Gliomen
Greek	Φωσφοροθειικό ολιγονουκλεοτίδιο (<i>TAAACGTTATAACGTTATGACGTCAT</i>)	Θεραπεία του γλοιώματος
Hungarian	Foszfor-tioát oligonukleotid (<i>TAAACGTTATAACGTTATGACGTCAT</i>), nátrium sója	Glioma kezelése
Italian	Oligonucleotide Fosforotioato (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sale sodico	Trattamento del glioma
Latvian	Fosfortioāta oligonukleotīds (<i>TAAACGTTATAACGTTATGACGTCAT</i>), nātrija sāls	Gliomas ārstēšana
Lithuanian	Fosforotioato oligonukleotidas (<i>TAAACGTTATAACGTTATGACGTCAT</i>), natrio druska	Gliomos gydymas
Polish	Oligonukleotyd fosforylowany (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sól sodowa	Leczenie glejaka
Portuguese	Oligonucleotido fosforotioato (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sal sódico	Tratamento do glioma
Slovak	Fosforotioát oligonukleotid (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sodná soľ	Liečba gliómu

Slovenian	Fosforotioatni oligonukleotidi (<i>TAAACGTTATAACGTTATGACGTCAT</i>); natrijeva sol	Zdravljenje glioma
Spanish	Oligonucleótido fosforotioato (<i>TAAACGTTATAACGTTATGACGTCAT</i>), sal sódica	Tratamiento del glioma
Swedish	Fosforotioatoligonukleotid (<i>TAAACGTTATAACGTTATGACGTCAT</i>), natrium salt	Behandling av gliom
Norwegian	Fosfortioatoligonukleotid, (<i>TAAACGTTATAACGTTATGACGTCAT</i>), natriumsalt	Behandling av gliom
Icelandic	Fosfórtíóat ólígónúkleótíð (<i>TAAACGTTATAACGTTATGACGTCAT</i>), nátríumsalt	Meðhöndlun á glíóma