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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

16-Base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide for the treatment of medulloblastoma

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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 1 October 2010, orphan designation (EU/3/10/789) was granted by the European Commission to Biogenera srl, Italy, for 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide for the treatment of medulloblastoma.

In March 2014, Biogenera srl changed name to Biogenera SpA.

What is medulloblastoma?

Medulloblastoma is a type of brain tumour that mainly affects children between the ages of three and eight years. The tumour develops in the cerebellum (a region at the bottom of the brain), but may spread to other parts of the brain. The first symptoms of the disease are usually caused by the increased pressure within the skull, and include nausea (feeling sick) and vomiting, headache, ataxia (inability to co-ordinate muscle movements) and irritability.

Medulloblastoma is a debilitating and life-threatening disease because it severely damages the brain and is associated with poor long-term survival.

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

At the time of designation, medulloblastoma affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 people*, and is below the

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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threshold 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 designation, the main treatment for medulloblastoma was surgery to remove the tumour. This was usually followed by radiotherapy (treatment with radiation, which was only used in children above three years of age) and chemotherapy (medicines to treat cancer), to reduce the risk of the tumour coming back. Two anticancer medicines vincristine and carboplatin were specifically authorised for medulloblastoma in the EU.

The sponsor has provided sufficient information to show that the medicine '16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide' might be of significant benefit for patients with medulloblastoma because it works in a different way to existing treatments and early studies in experimental models indicate that it might be used in combination with existing treatments to improve the outcome of patients with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

16-Base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide is expected to work by attaching to and blocking the gene responsible for producing a protein called MYCN. This protein is involved in the growth, progression and spread of medulloblastoma cells. By blocking the gene, the medicine blocks the production of the MYCN protein. This is expected to stop the medulloblastoma cells from growing and multiplying.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide in experimental models was ongoing.

At the time of submission, no clinical trials with this medicine in patients with medulloblastoma had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for medulloblastoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2010 recommending the granting of this designation.

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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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide	Treatment of medulloblastoma
Bulgarian	Едноверижен Пептид Нуклеинова Киселина олигонуклеотид от 16 бази, свързан с7 аминокиселинен пептид	Лечение на медулобластом
Czech	Jednořetězcový peptid nukleotidové kyseliny oligonukleotid o 16 bazích vázaný na 7 aminokyselinový peptid	Léčba medulloblastomu
Danish	16 baser enkeltstrenget peptidnukleinsyre oligonukleotid bundet til et 7 aminosyre peptid	Behandling af medulloblastom
Dutch	16-base enkelstrandig peptide nucleïnezuur oligonucleotide geliëerd aan een 7- aminozuren bevattend peptide	Behandeling van medulloblastoom
Estonian	16 alusega üheahelalise peptiid- nukleiinhappe oligonukleotiid, mis on seotud 7-aminohappe peptiidiga	Medulloblastoomi ravi
Finnish	16-emäksinen yksijuosteinen peptidinukleiinihappo-oligonukleotidi - 7 aminohappopeptidi	Medulloblastooman hoito
French	Oligonucléotide peptide acide nucléique monobrin de 16 bases lié à un peptide de 7 aminoacides	Traitement du médulloblastome
German	16-Basen Einzelstrang Peptid-Nucleinsäure-Oligonukleotid gebunden an ein 7 Aminosäure Peptid	Behandlung des Medulloblastoms
Greek	Ολιγονουκλεοτίδιο μονής αλύσου 16 βάσεων συζευγμένο με πεπτίδιο 7 αμινοξέων	Θεραπεία Μεδουλοβλαστώματος
Hungarian	7 aminosavból álló peptidhez kapcsolt 16 bázisból álló, egyes szálú peptid-nukleinsav oligonukleotid	Medulloblastoma kezelése
Italian	Oligonucleotide peptidico a catena singola di 16 basi connesso a oligopeptide di 7 aminoacidi	Trattamento del medulloblastoma
Latvian	16 bāzu vienpavediena peptīda nukleīnskābes oligonukleotīda - 7 aminoskābju peptīds	Meduloblastomas ārstēšana
Lithuanian	Viengrandis 16 bazių peptido nukleorūgšties oligonukleotidas, prijungtas prie 7 aminorūgščių peptidopeptide	Meduloblastomos gydymas
Maltese	Oligonukleotide tal-aċidu nukleiku peptidiku b'katina waħda ta' 16 il-bażi magħqud ma' peptidu b' 7 aċidi amminiċi	Kura tal-medulloblastoma
Polish	16 zasadowy jednoniciowy oligonukleotyd kwasu peptydonukleinowego przyłączony do 7-aminokwasowego peptydu	Leczenie rdzeniaka płodowego

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Oligonucleótido monocatenário de ácido nucleico peptídico com 16 bases ligado a um péptido de 7 aminoácidos	Tratamento do medulloblastoma
Romanian	Oligonucleotid de acid peptidonucleic monocatenar format din 16 baze, cuplat cu un peptid format din 7 aminoacizi	Tratamentul meduloblastomului
Slovak	Jednovláknový oligonukleotid peptidovej nukleovej kyseliny zložený zo 16 báz vo väzbe so 7-aminokyselinovým peptidom	Liečba meduloblastómu
Slovenian	16 bazni enoverižni peptid nukleinske kisline oligonukleotid – vezan na 7 aminokislinski peptid	Zdravljenje nevroendokrinega meduloblastoma
Spanish	Oligonucleótido monocatenario de ácido nucleico peptídico compuesto por 16 bases ligado a un péptido de 7 aminoácidos	Tratamiento del meduloblastoma
Swedish	16-bas enkelsträngad peptidnukleinsyra-oligonukleotid länkt med 7 aminosyra peptid	Behandling av medulloblastom
Norwegian	16-baser enkelttrådet peptidnukleinsyre-oligonukleotid koblet til et 7 aminosyrepeptid	Behandling av medulloblastom
Icelandic	16 basa einþátta peptíð kjarnasýru ólígónúkleótíð - 7 amínósýru peptíð	Meðferð við mænukímfrumuæxli