



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 March 2014
EMA/COMP/393098/2012 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

16-base single-stranded peptide nucleic acid oligonucleotide linked to 7-amino acid peptide for the treatment of neuroblastoma

First publication	19 July 2012
Rev.1: sponsor's name change	24 March 2014
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 4 July 2012, orphan designation (EU/3/12/1016) was granted by the European Commission to Biogenera srl, Italy, for 16-base single-stranded peptide nucleic acid oligonucleotide linked to 7-amino acid peptide for the treatment of neuroblastoma.

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

What is neuroblastoma?

Neuroblastoma is a cancer of nerve cells which is usually seen as a lump in the abdomen (belly area) or around the spine. Symptoms may include weakness, bone pain, loss of appetite and fever. Neuroblastoma is the most common solid tumour outside the brain in children. In many cases it is present at birth but is diagnosed later when the cancer has spread to other parts of the body and the child begins to show symptoms of the disease.

Neuroblastoma is a long-term debilitating and life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, neuroblastoma affected not more than 0.18 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 9,200 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 509,000,000 (Eurostat 2012).



ceiling 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, several medicines were authorised in the EU for the treatment of neuroblastoma. Treatments for neuroblastoma included surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with neuroblastoma because early studies in experimental models show that it works in a different way to existing medicines and might be used in addition to other treatments. 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?

This medicine is expected to work by attaching to and blocking the gene responsible for producing a protein called N-Myc, which is involved in the growth, progression and spread of neuroblastomas. By blocking the gene, the medicine blocks the production of the N-Myc protein. This is expected to stop the neuroblastoma 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 the medicine in experimental models was ongoing.

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

At the time of submission, the medicine was not authorised anywhere in the EU for neuroblastoma 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 11 May 2012 recommending the granting of this 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;
- 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	16-base single-stranded peptide nucleic acid oligonucleotide linked to 7-amino acid peptide	Treatment of neuroblastoma
Bulgarian	Едноверижен пептиден олигонуклеотид от 16-бази, свързан със 7-аминокиселинен пептид	Лечение на невробластом
Czech	Jednořetězcový peptid nukleové kyseliny oligonukleotid o 16-bázích navázaný na heptapeptid	Léčba neuroblastomu
Danish	16-baser enkeltstrenget peptidnukleinsyre oligonukleotid 7-aminosyre peptid	Behandling af neuroblastom
Dutch	16-base enkelstrengig peptide nucleïnezuur oligonucleotide 7-aminozurenpeptide	Behandeling van neuroblastoom
Estonian	16-alusega üheaahelalise peptiid- nukleinhappe oligonukleotiid, mis on seotud 7-aminohappe peptiidiga	Neuroblastoomi ravi
Finnish	16-emäksinen yksijuosteinen peptidinukleiinihappo-oligonukleotidi 7-aminohappopeptidi	Neuroblastooman hoito
French	Oligonucléotide monobrin d'acide nucléique peptide à 16-bases lié à un peptide de 7-aminoacides	Traitement du neuroblastome
German	16-Basen Einzelstrang-Peptidnukleinsäure Oligonukleotid gebunden an ein 7-Aminosäure Peptid	Behandlung des Neuroblastoms
Greek	Ολιγονουκλεοτίδιο μονής αλύσου 16-βάσεων συζευγμένο με πεπτίδιο 7-αμινοξέων	Θεραπεία Νευροβλάστωματος
Hungarian	7-aminosavból álló peptidhez kapcsolt 16-bázisból álló, egyes szálú peptid-nukleinsav oligonukleotid	Neuroblastoma kezelése
Italian	Oligonucleotide peptidico a catena singola di 16-basi connesso a oligopeptide di 7-aminoacidi	Trattamento del neuroblastoma
Latvian	16-bāžu vienpavediena peptīda nukleīnskābes oligonukleotīda 7-aminoskābju peptīds	Neiroblastomas ārstēšana
Lithuanian	16 bazių viengrandžio peptido nukleorūgšties oligonukleotido-7 aminorūgščių peptidas	Neuroblastomos gydymas
Maltese	Oligonukleotide tal-aċidu nukleiku peptidiku b'katina waħda ta' 16 il-baži magħqud ma' peptidu b' 7-aċidi amminiċi	Kura tan-newroblastoma
Polish	16-zasadowy jednoniciowy oligonukleotyd kwasu peptydonukleinowego przyłączony do 7-aminokwasowego peptydu	Leczenie nerwiaka płodowego
Portuguese	Oligonucléotido monocatenário de 16-ácidos nucleicos ligado a um péptido de 7-amino ácidos	Tratamento do neuroblastoma
Romanian	Peptid monocatenar 16-baze acid nucleic oligonucleotid-peptid 7-aminoacizi	Tratamentul neuroblastomului

¹ At the time of designation

Language	Active ingredient	Indication
Slovak	Oligonukleotid jednovláknovej peptidovej nukleovej kyseliny zložený zo 16-báz vo väzbe so 7-aminokyselinovým peptidom	Liečba neuroblastómu
Slovenian	Peptidni 16-bazni enoverižni oligonukleotid s 7-aminokislinskim peptidom	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Oligonucleótido monocatenario de ácido nucleico peptídico compuesto por 16-bases ligado a un péptido de 7-aminoácidos	Tratamiento del neuroblastoma
Swedish	16-bas enkelsträngad peptidnukleinsyra-oligonukleotid kopplat till en 7-aminosyra peptid	Behandling av neuroblastom
Norwegian	16-baser enkelttrådet peptidnukleinsyre-oligonukleotid koblet til et 7 aminosyrepeptid	Behandling av neuroblastom
Icelandic	16 basa einpátta peptíð kjarnasýru ólígónúkleótíð - 7 amínósýru peptíð	Meðferð við taugakímfrumuæxli