

5 September 2016
EMA/COMP/451052/2016
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 soft tissue sarcoma

On 14 July 2016, orphan designation (EU/3/16/1690) was granted by the European Commission to Biogenara SpA, Italy, for 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7 amino acid peptide for the treatment of soft tissue sarcoma.

What is soft tissue sarcoma?

Soft tissue sarcoma is a cancer that affects the soft, supportive tissues of the body. It can occur in muscles, blood vessels, fat tissue or in other tissues that support, surround and protect organs. Patients with soft tissue sarcoma do not usually have symptoms in the early stages of the disease. First symptoms appear when the tumour grows large enough to cause swelling and pain.

Soft tissue sarcoma is a long-term debilitating and life-threatening disease, particularly when the cancer has spread to other parts of the body.

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

At the time of designation, soft tissue sarcoma affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 134,000 people*, and is below the 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, the main treatment for early-stage soft tissue sarcoma was surgery. For large sarcomas, surgery was usually followed by radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer) to kill any cancer cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma including doxorubicin.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with soft tissue sarcoma because it works in a different way to other treatments and studies in experimental models indicate that it might reduce tumour size. 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 soft tissue sarcoma. By blocking the gene, the medicine prevents the production of the N-Myc protein. This is expected to stop the cancer cells from growing and multiplying.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with soft tissue sarcoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for soft tissue sarcoma 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 16 June 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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 a 7 aminoacid peptide	Treatment of soft tissue sarcoma
Bulgarian	Едноверижен пептиден олигонуклеотид от 16-бази, свързан със 7-аминокиселинен пептид	Лечение на сарком на меките тъкани
Croatian	Oligonukleotidni jednolančani peptid od 16 baza vezan za peptid od 7 aminokiselina	Liječenje sarkoma mekih tkiva
Czech	Jednořetězcový peptid nukleové kyseliny oligonukleotid o 16-bázích navázaný na heptapeptid	Léčba sarkomu měkkých tkání
Danish	16-baser enkeltstrenget peptidnukleinsyre oligonukleotid 7-aminosyre peptid	Behandling af bløddelssarkom
Dutch	16-base enkelstrengig peptide nucleïnezuur oligonucleotide 7-aminozurenpeptide	Behandeling weke delen sarcoom
Estonian	16-alusega üheaheelalise peptiid- nukleiinhappe oligonukleotiid, mis on seotud 7-aminohappe peptiidiga	Pehmele kudede sarkoomi ravi
Finnish	16-emäksinen yksijuosteinen peptidinukleiinihappo-oligonukleotidi 7-aminohappopeptidi	Pehmytkudossarkooman hoito
French	Oligonucléotide monobrin d'acide nucléique peptide à 16-bases lié à un peptide de 7-aminoacides	Traitement des sarcomes des tissus mous
German	16-Basen Einzelstrang-Peptidnukleinsäure Oligonukleotid gebunden an ein 7-Aminosäure Peptid	Behandlung des Weichteilsarkoms
Greek	Ολιγονουκλεοτίδιο μονής αλύσου 16-βάσεων συζευγμένο με πεπτίδιο 7-αμινοξέων	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	7-aminosavból álló peptidhez kapcsolt 16-bázisból álló, egyes szálú peptid-nukleinsav oligonukleotid	Lágy szöveti sarcoma kezelése
Italian	Oligonucleotide peptidico a catena singola di 16-basi connesso a oligopeptide di 7-aminoacidi	Trattamento dei sarcomi dei tessuti molli
Latvian	16-bāžu vienpavediena peptīda nukleīnskābes oligonukleotīd, kas saistīts ar 7-aminoskābju peptīdu	Mīksto audu sarkomas ārstēšana
Lithuanian	16 bazių viengrandžio peptido nukleorūgšties oligonukleotido-7 aminorūgščių peptidas	Minkštųjų audinių sarkomos gydymas
Maltese	Oligonukleotide tal-aċidu nukleiku peptidiku b'katina waħda ta' 16 il-baži magħqud ma' peptidu b' 7-aċidi amminiċi	Kura tas-sarkoma tat-tessuti rotob
Polish	16-zasadowy jednoniciowy oligonukleotyd kwasu peptydonukleinowego przyłączony do 7-aminokwasowego peptydu	Leczenie mięsaków tkanek miękkich
Portuguese	Oligonucleótido monocatenário de 16-ácidos nucleicos ligado a um péptido de 7-amino ácidos	Tratamento do sarcoma dos tecidos moles
Romanian	Peptid monocatenar 16-baze acid nucleic oligonucleotid-peptid 7-aminoacizi	Tratamentul sarcomului țesuturilor moi

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 sarkómu mäkkých tkanív
Slovenian	Peptidni 16-bazni enoverižni oligonukleotid s 7-aminokislinskim peptidom	Zdravljenje sarkoma mehkih tkiv
Spanish	Oligonucleótido monocatenario de ácido nucleico peptídico compuesto por 16-bases ligado a un péptido de 7-aminoácidos	Tratamiento del sarcoma de tejidos blandos
Swedish	16-bas enkelsträngad peptidnukleinsyra-oligonukleotid kopplat till en 7-aminosyra peptid	Behandling av mjukdelssarkom
Norwegian	16-baser enkelttrådet peptidnukleinsyre-oligonukleotid koblet til et 7 aminosyrepeptid	Behandling av bløtvevssarkom
Icelandic	16 basa einpátta peptíð-jarnasýru ólígkleótíð -7 amínósýru peptíð	Meðferð við mjúkvefjasarkmeini