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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Glucopyranosyl lipid A stable emulsion and recombinant New York oesophageal squamous cell carcinoma-1 protein for the treatment of soft tissue sarcoma

On 21 March 2016, orphan designation (EU/3/16/1634) was granted by the European Commission to Pharm Research Associates (UK) Limited, United Kingdom, for glucopyranosyl lipid A stable emulsion and recombinant New York oesophageal squamous cell carcinoma-1 protein (NY-ESO-1) for the treatment of soft tissue sarcoma.

What is soft tissue sarcoma?

Soft tissue sarcoma is a type of 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 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 118,000 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

*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).

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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.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with soft tissue sarcoma because early studies suggest that it may be of benefit in patients previously given other authorised cancer medicines. 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?

The medicine works by stimulating the patient's immune system, the body's natural defences, so that it targets and destroys the cancer cells. It contains a protein called NY-ESO-1, which is found at high levels on cells in many soft tissue sarcomas. This is combined with a compound that helps to stimulate the immune system. When the medicine is given, the patient's immune system is expected to learn to treat NY-ESO-1 as 'foreign'. This is expected to stimulate an immune response against the cancer cells carrying NY-ESO-1 on their surface, resulting in the immune system attacking and destroying the cancer cells.

The medicine can be used together with another medicine (called 'Sindbis virus envelope pseudotyped lentiviral vector encoding New York oesophageal squamous cell carcinoma-1 protein') that helps the immune system to recognise the NY-ESO-1 protein as foreign and so stimulate the body to attack the cancer.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with soft tissue sarcoma were ongoing.

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 18 February 2016 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein	Treatment of soft tissue sarcoma
Bulgarian	Глюкопиранозил липид А стабилна емулсия и рекомбинантен нюйоркски езофагеален плоскоклетъчен карцином-1	Лечение на сарком на меките тъкани
Croatian	Glukopiranozil lipid A stabilna emulzija i rekombinantni antigen NY-ESCC (planocelularni karcinom jednjaka)-1	Liječenje sarkoma mekih tkiva
Czech	Stabilní emulze glukopyranosyl lipidu A a rekombinantního New York antigenu 1 ezofageálního dlaždicobuněčného karcinomu	Léčba sarkomu měkkých tkání
Danish	Glucopyranosyl lipid A stabil emulsion og rekombinant New York øsofagus planocellulært karcinom-1	Behandling af bløddelssarkom
Dutch	Stabiele glucopyranosyl lipide A emulsie en recombinant New York oesofageaal levyepiteelikarsinoomasolu 1	Behandeling weke delen sarcoom
Estonian	Glükopüranosüül lipiid A stabiilne emulsioon ja rekombinantne New Yorgi ösofageaalne lamerakkvähk-1	Pehmete kudede sarkoomi ravi
Finnish	Glukopyranosyyli lipidi A:n stabiili emulsio ja rekombinantti New York ruokatorven levyepiteelikarsinooma 1	Pehmytkudossarkooman hoito
French	Émulsion stable de lipide A glucopyranosylique et de protéines recombinantes New York 1 de carcinome épidermoïde de l'œsophage	Traitement des sarcomes des tissus mous
German	Stabile Emulsion von Glucopyranosyl Lipid und rekombinantes New York Ösophagus-Plattenepithelkarzinom-1	Behandlung des Weichteilsarkoms
Greek	Σταθερό γαλάκτωμα γλυκοπυρανοσυλικού λιπιδίου Α και ανασυνδυασμένο αντιγόνο Νέας Υόρκης του ακανθοκυτταρικού καρκινώματος οισοφάγου-1.	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Glükopiranozil-lipid-A stabil emulziója és rekombináns New York oesophagealis squamosus sejtes carcinoma-1 fehérje	Lágy szöveti sarcoma kezelése
Italian	Emulsione stabile di glucopiranosile lipide A e protiena ricombinante New York-1 del carcinoma esofageo a cellule squamose	Trattamento dei sarcomi dei tessuti molli
Latvian	Glikopiranozila A lipīda stabila emulsija un rekombinants Ņujorkas barības vada plakano šūnu karcinomas 1 proteīns	Mīksto audu sarkomas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Stabili gliukopiranozilo lipido A emulsija ir rekombinantinė Niujorko plokščialąstelinė stemplės karcinoma-1	Minkštųjų audinių sarkomos gydymas
Maltese	Emulsjoni stabbli ta' glucopyranosyl lipid A u karċinoma taċ-ċelluli skwamużi tal-esofagu New York 1 rikombinanti	Kura tas-sarkoma tat-tessuti rotob
Polish	Trwała emulsja adjuwantu glukopiranozylolipidowego i rekombinowany antygen raka płaskokomórkowego przełyku New York 1	Leczenie mięsaków tkanek miękkich
Portuguese	Emulsão estável de lípidos A glucopiranosil e carcinoma-1 de células escamosas do esófago New York recombinante	Tratamento do sarcoma dos tecidos moles
Romanian	Emulsie stabilă de glucopiranozil lipid A și proteină recombinantă de carcinom esofagian cu celule scuamoase New-York-1	Tratamentul sarcomului ţesuturilor moi
Slovak	Glukopyranozyl lipid A – stabilná emulzia a rekombinantný antigén spinocelulárneho karcinómu pažeráka typu New York 1	Liečba sarkómu mäkkých tkanív
Slovenian	Stabilna emulzija glukopiranozil lipida A in rekombinantnega antigena New York 1 ploščatoceličnega raka požiralnika	Zdravljenje sarkoma mehkih tkiv
Spanish	Emulsión estable de glucopiranosil lípido A y carcinoma esofágico de células escamosas Nueva York 1 recombinante	Tratamiento del sarcoma de tejidos blandos
Swedish	Glukopyranosyl lipid A stabil emulsion och rekombinant New York esofagus skivepitelcancer-1	Behandling av mjukdelssarkom
Norwegian	Glukopyranosyl lipid A stabil emulsjon og rekombinant New York esophageal esofageal -1	Behandling av bløtvevssarkom
Icelandic	Stöðugt glúkópýranósýl lípíð-A fleyti og New York-vélindakrabbameinsmótefnavaki-1 sem framleiddur er með erfðatækni	Meðferð við mjúkvefjasarkmeini