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EMA/COMP/303494/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor for the treatment of soft tissue sarcoma

On 19 June 2013, orphan designation (EU/3/13/1145) was granted by the European Commission to Oncos Therapeutics Oy, Finland, for genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor for the treatment of soft tissue sarcoma.

What is soft tissue sarcoma?

Soft tissue sarcomas are a type of cancer that affect the soft, supportive tissues of the body. They 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 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 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 152,000 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 cancerous cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma.

*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. This represents a population of 509,000,000 (Eurostat 2013).

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The sponsor has provided sufficient information to show that genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor might be of significant benefit for patients with soft tissue sarcoma because early studies suggest that it may make treatment of the tumour more effective when it is combined with existing 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?

This medicine contains a virus that has been modified by the insertion of a gene so that it can produce a protein called 'granulocyte-macrophage colony-stimulating factor' (GM-CSF). It has also had one of its own genes inactivated so it can only reproduce itself within cancer cells. When the medicine is injected into a patient with soft tissue sarcoma, the virus is expected to selectively attach to the cancer cells, enter them and reproduce inside them, eventually killing them. In addition, it is expected to produce the GM-CSF protein, which stimulates the immune system (the body's natural defences) to attack the cancerous cells.

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, 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 15 May 2013 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	Genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor	Treatment of soft tissue sarcoma
Bulgarian	Генетично модифициран аденовирус серотип 5/3, кодиращ гранулоцит макрофаг колония-стимулиращ фактор	Лечение на сарком на меките тъкани
Czech	Geneticky modifikovaný adenovirus sérotypu 5/3 kódující faktor stimulující kolonie granulocytů a makrofágů.	Léčba sarkomu měkkých tkání
Danish	Genetisk modificeret serotype 5/3-adenovirus, der koder for granulocyt/makrofag-kolonistimulerende faktor	Behandling af bløddelssarkom
Dutch	Genetisch gemodificeerd adenovirus serotype 5/3 dat codeert voor granulocyt-macrofaag koloniestimulerende factor	Behandeling van weke delen sarcoom
Estonian	Granulotsüütide-makrofaagide kolooniaid stimuleerivat faktorit kodeeriv adenoviiruse geneetiliselt muundatud serotüüp 5/3	Pehmete kudede sarkoomi ravi
Finnish	Granulosyytti-makrofagikasvutekijää koodaava serotyypin 5/3 muuntogeeninen adenovirus	Pehmytkudossarkooman hoito
French	Adénovirus de sérotype 5/3 génétiquement modifié codant pour le facteur de croissance des granulocytes-macrophages	Traitement des sarcomes des tissus mous
German	Genetisch modifiziertes Adenovirus vom Serotyp 5/3, das für den Granulozyten-Makrophagen koloniestimulierenden Faktor kodiert	Behandlung des Weichteilsarkoms
Greek	Γενετικά τροποποιημένος αδενοϊός ορότυπου 5/3 που κωδικοποιεί τον παράγοντα διέγερσης αποικιών κοκκιοκυττάρων-μακροφάγων	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Granulocyta-makrofág kolóniastimuláló faktort kódoló, genetikailag módosított 5/3-as szerotípusú adenovírus	Lágy szöveti sarcoma kezelése
Italian	Adenovirus di sierotipo 5/3 geneticamente modificato che codifica per il fattore stimolante le colonie di granulociti e macrofagi	Trattamento dei sarcomi dei tessuti molli
Latvian	Ģenētiski modificēta serotipa 5/3 adenovīrusa kodējums granulocītu makrofāgu koloniju stimulējošam faktoram	Mīksto audu sarkomas ārstēšana
Lithuanian	Genetiškai modifikuoti serotipo 5/3 adenovirusai, koduojantys granuliocitų makrofagų kolonijas stimuliuojantį faktorių	Minkštųjų audinių sarkomos gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Adenovirus ta' serotip 5/3 modifikat ġenetikament li jikkodifika għall-fattur li jistimula kolonji makrofagu-granuloċita	Kura tas-sarkoma tat-tessuti rotob
Polish	Genetycznie zmodyfikowany adenowirus serotypu 5/3 kodujący czynnik stymulujący wzrost kolonii granulocytów i makrofagów	Leczenie mięsaków tkanek miękkich
Portuguese	Adenovírus de serotipo 5/3 geneticamente modificado codificando o fator estimulador de colónias de granulócitos e macrófagos	Tratamento do sarcoma dos tecidos moles
Romanian	Adenovirus serotip 5/3 modificat genetic pentru a codifica factorul stimulator al coloniilor de granulocite macrofage	Tratamentul sarcomului ţesuturilor moi
Slovak	Geneticky modifikovaný adenovírus sérotypu 5/3 kódujúci faktor stimulujúci kolónie granulocytov a makrofágov	Liečba sarkómu mäkkých tkanív
Slovenian	Genetsko spremenjeni adenovirus serotipa 5/3, kodiran za spodbujajoči faktor kolonij granulocitnih makrofagov	Zdravljenje sarkoma mehkih tkiv
Spanish	Adenovirus de serotipo 5/3 genéticamente modificado que codifica el factor estimulante de colonias de granulocitos-macrófagos	Tratamiento del sarcoma de tejidos blandos
Swedish	Genetiskt modifierat adenovirus serotyp 5/3 som kodar för granulocyt-makrofagkolonistimulerande faktor	Behandling av mjukdelssarkom
Norwegian	Genetisk modifisert adenovirus serotype 5/3 som koder for granulocytt-makrofag-kolonistimulerende faktor	Behandling av bløtvevssarkom
Icelandic	Erfðabreytt adenóveira af sermisgerð 5/3 sem kóðar fyrir GM-CSF vaxtarþætti	Meðferð við mjúkvefjasarkmeini