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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 ovarian cancer

On 29 April 2014, orphan designation (EU/3/14/1265) 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 ovarian cancer.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries, the two organs in the female reproductive system that produce eggs. Most ovarian cancers occur in women over the age of 50 years. Due to the absence of symptoms in the early stages of the disease, the majority of patients are diagnosed when the cancer has spread to other parts of the body.

Ovarian cancer 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, ovarian cancer affected less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 153,000 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, several medicines were authorised in the EU for the treatment of ovarian cancer. The choice of treatment depended mainly on how advanced the disease was. Treatments included surgery and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ovarian cancer because early studies in patients suggest that it might improve

^{*}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 511,100,000 (Eurostat 2014).

the condition of patients with ovarian cancer resistant to treatment with existing therapies. 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 into which a gene has been inserted 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 ovarian cancer, 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 in cancer cells, 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 ovarian cancer were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for ovarian cancer 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 12 March 2014 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	Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor	Treatment of ovarian cancer
Bulgarian	Генетично модифициран аденовирус серотип 5/3, кодиращ гранулоцит макрофаг колония-стимулиращ фактор	Лечение на рак на яйчниците
Croatian	Genetski modificiran adenovirus serotipa 5/3 koji kodira faktor stimulacije rasta granulocitno-makrofagnih kolonija	Liječenje raka jajnika
Czech	Geneticky modifikovaný adenovirus sérotypu 5/3 kódující faktor stimulující kolonie granulocytů a makrofágů	Léčba karcinomu vaječníků
Danish	Genetisk modificeret serotype 5/3-adenovirus, der koder for granulocyt/makrofag-kolonestimulerende faktor	Behandling af ovarie cancer
Dutch	Genetisch gemodificeerd adenovirus serotype 5/3 dat codeert voor granulocyt-macrofaag kolonestimulerende factor	Behandeling van ovariumkanker
Estonian	Granulotsüütide-makrofaagide kolooniaid stimuleerivat faktorit kodeeriv adenoviiruse geneetiliselt muundatud serotüüp 5/3	Munasarjavähi ravi
Finnish	Granulosyytti-makrofagikasvutekijää koodaava serotyypin 5/3 muuntogeeninen adenovirus	Munasarjasyövän hoito
French	Adénovirus de sérotype 5/3 génétiquement modifié codant pour le facteur de croissance des granulocytes-macrophages	Traitement du cancer de l'ovaire
German	Genetisch modifiziertes Adenovirus vom Serotyp 5/3, das für den Granulozyten-Makrophagen kolonestimulierenden Faktor kodiert	Behandlung des Ovarialkarzinoms
Greek	Γενετικά τροποποιημένος αδενοϊός ορότυπου 5/3 που κωδικοποιεί τον παράγοντα διέγερσης αποικιών κοκκιοκυττάρων-μακροφάγων	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Granulocytá-makrofág kolóniastimuláló faktort kódoló, genetikailag módosított 5/3-as szerotípusú adenovírus	Petefészekrák kezelése
Italian	Adenovirus di sierotipo 5/3 geneticamente modificato che codifica per il fattore stimolante le colonie di granulociti e macrofagi	Trattamento del carcinoma dell'ovaio
Latvian	Ģenētiski modificēts 5/3 serotipa adenovīruss, kas kodē granulocītu makrofāgu koloniju stimulējošo faktoru	Olnīcu vēža ārstēšana

¹ At the time of designation

Language	Active ingredient	Indication
Lithuanian	Genetiškai modifikuotas serotipo 5/3 adenovirusas, koduojantis granulocitų makrofagų kolonijas stimuliuojantį faktorių	Kiaušidžių vėžio gydymas
Maltese	Adenovirus ta' serotip 5/3 modifikat ġenetikament li jikkodifika għall-fattur li jstimula kolonji makrofagu-granuloċita	Kura tal-kanċer ta' l-ovarji
Polish	Genetycznie zmodyfikowany adenowirus serotypu 5/3 kodujący czynnik stymulujący wzrost kolonii granulocytów i makrofagów	Leczenie raka jajnika
Portuguese	Adenovírus de serotipo 5/3 geneticamente modificado codificando o fator estimulador de colónias de granulócitos e macrófagos	Tratamento do carcinoma do ovário
Romanian	Adenovirus serotip 5/3 modificat genetic pentru a codifica factorul de stimulare al coloniilor formatoare de granulocite macrofage	Tratamentul cancerului ovarian
Slovak	Geneticky modifikovaný adenovírus sérotypu 5/3 kódujúci faktor stimulujúci kolónie granulocytov a makrofágov	Liečba rakoviny vaječníkov
Slovenian	Genetsko spremenjeni adenovirus serotipa 5/3, kodiran za spodbujajoči faktor kolonij granulocitnih makrofagov	Zdravljenje raka na jajčnikih
Spanish	Adenovirus de serotipo 5/3 genéticamente modificado que codifica el factor estimulante de colonias de granulocitos-macrófagos	Tratamiento del cáncer de ovario
Swedish	Genetiskt modifierat adenovirus serotyp 5/3 som kodar för granulocyt-makrofagkolonistimulerande faktor	Behandling av ovarialcancer
Norwegian	Genetisk modifisert adenovirus serotype 5/3 som koder for granulocyt-makrofag-kolonistimulerende faktor	Behandling av eggstokkreft
Icelandic	Erfðabreytt adenóveira af sermisgerð 5/3 sem kóðar fyrir GM-CSF vaxtarþætti	Meðferð eggjastokkakrabbameins