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Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo for the treatment of ovarian cancer

On 16 April 2018, orphan designation (EU/3/18/2009) was granted by the European Commission to SOTIO a.s, Czech Republic, for autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo (also known as DCVAC/OvCa) 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 aged over 50 years. Due to the absence of clear symptoms in the early stages of the disease, the majority of women are diagnosed when the cancer has spread to other parts of the body.

Ovarian cancer is a 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 approximately 4.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 243,000 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 this medicine might be of significant benefit for patients with ovarian cancer. Preliminary results from studies show that the medicine may

*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 517,400,000 (Eurostat 2018).

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increase the length of time patients live without their disease getting worse when added to standard chemotherapy.

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 cells of the immune system (the body's natural defences) to attack cancer cells. It is made from the patient's own dendritic cells (a type of immune cell), whose job is to stimulate other immune cells to attack foreign bodies. Before being injected into the body, the dendritic cells are grown outside the body and mixed with killed ovarian cancer cells to make them better at stimulating the immune cells to attack the cancer 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. Orphan designation had been granted in the United States for ovarian cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 March 2018 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Autologous dendritic cells pulsed with killed ovarian cancer cells and matured by TLR3 ligand ex vivo	Treatment of ovarian cancer
Bulgarian	Автоложни дендритни клетки, обработени пулсово с убити овариални ракови клетки и матурирани ex vivo посредством TLR3-лиганд	Лечение на рак на яйчниците
Croatian	Autologne dendritične stanice pulsirane usmrcenim stanicama raka jajnika sazrijevane s TLR3 ligandom u ex vivo uvjetima	Liječenje raka jajnika
Czech	Autologní dendritické buňky pulzované usmrcenými ovariálními nádorovými buňkami a maturované ligandem TLR3 ex vivo	Léčba karcinomu vaječníků
Danish	Autologe dendritiske celler primet med inaktive ovariecancerceller, og lagret med TLR3 ligand ex vivo	Behandling af ovarie cancer
Dutch	Autologe dendritische cellen gepulseerd met gedode eierstokkankercellen en gerijpt door TLR3 ligand ex vivo	Behandeling van ovariumkanker
Estonian	Kehaväliselt munasarjavähi surmatud rakkudega aktiveeritud ja TLR3 ligandiga täiskasvanud staadiumisse viidud autoloogsed dendriitrakud	Munasarjavähi ravi
Finnish	Autologiset dendriittisolut ladattuina tapetuilla munasarjasyövän soluilla, ja kypsytetty TLR3 ligandin kanssa ex vivo	Munasarjasyövän hoito
French	Cellules dendritiques autologues pulsées avec des cellules cancéreuses mortes de l'ovaire et rendues matures grâce au ligand TLR3 ex vivo	Traitement du cancer de l'ovaire
German	Autologe dendritische Zellen, gepulst mit abgetöten Ovarialkarzinom-Zellen, und ex vivo g e r e i f t mittels TLR3 Ligand	Behandlung des Ovarialkarzinoms
Greek	Αυτόλογα δενδριτικά κύτταρα επωασμένα με νεκρά κύτταρα καρκίνου των ωοθηκών και καλλιεργημένων ex vivo παρουσία συνδέτη TLR3	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Elölt ovárium karcinóma sejtekkel stimulált és TLR3 ligandokkal ex vivo érlelt autológ dendritikus sejtek	Petefészekrák kezelése
Italian	Cellule dendritiche autologhe pulsate con cellule tumorali ovariche uccise, maturate ex vivo tramite il ligando TLR3	Trattamento del carcinoma dell'ovaio
Latvian	Autologas dendrītiskās šūnas, kas pārslogotas ar nogalinātām olnīcu vēža šūnām un nobriedinātas ar TLR3 ligandu ex vivo	Olnīcu vēža ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Autologinės dendritinės ląstelės pulsuojančios su žuvusiomis kiaušidžių vėžio ląstelėmis ir brandintos ligando TLR3 ex vivo	Kiaušidžių vėžio gydymas
Maltese	Ċelluli dendritiċi awtologi pulsati b'ċelluli maqtula ta' tumur ovarju u mmaturati permezz ta' ligand TLR3 ex vivo	Kura tal-kanċer ta' l-ovarji
Polish	Autologiczne komórki dendrytyczne stymulowane martwymi komórkami raka jajnika i dojrzewające pod wpływem ligandu TLR3 ex vivo	Leczenie raka jajnika
Portuguese	Células dendríticas autólogas pulsadas com células mortas de carcinoma do ovário e maturadas, ex vivo, com o ligante TLR3	Tratamento do carcinoma do ovário
Romanian	Celule dendritice autologe pulsate cu celule moarte de cancer ovarian și maturate ex vivo cu ligand TLR3	Tratamentul cancerului ovarian
Slovak	Autológne dendritické bunky pulzované usmrtenými ovariálnymi nádorovými bunkami a maturované ligandom TLR3 ex vivo	Liečba rakoviny vaječníkov
Slovenian	Avtologne dendritične celice spodbujene z uničenimi celicami raka jajčnikov in zorjene s TLR3 ligandom ex vivo	Zdravljenje raka na jajčnikih
Spanish	Células dendríticas autólogas pulsadas con células de cáncer ovárico muertas y maduradas por el ligando TLR3 ex vivo	Tratamiento del cáncer de ovario
Swedish	Autologa dendritiska celler primade med döda äggstockscancerceller som mognat med en TLR3 ligand ex vivo	Behandling av ovarialcancer
Norwegian	Autologe dendrittiske celler pulset med inaktive eggstokkreft celler og modnet med TLR3 ligand ex vivo	Behandling av eggstokkreft
Icelandic	Samgena dendritic frumur primed með óvirkum eggjastokka krabbameinsfrumum, og þroskast með TLR3 sameind ex vivo	Meðferð eggjastokkakrabbameins