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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose for the treatment of ovarian cancer

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Rev.1: transfer of sponsorship	20 March 2012
Rev.2: administrative update	14 October 2012

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 20 September 2010, orphan designation (EU/3/10/776) was granted by the European Commission to Prima Biomed Europe Ltd, United Kingdom, for autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose for the treatment of ovarian cancer.

The sponsorship was transferred to Prima Biomed GmbH, Germany, in February 2012.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries (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 locally or to other parts of the body.

Ovarian cancer is a life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, ovarian cancer 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 threshold 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 because it works in a different way to existing treatments and because early studies show that it might improve the treatment of patients with this condition, particularly patients whose disease has come back after previous treatment. 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?

Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose is a cancer vaccine. It is expected to work by activating the patient's immune system (the body's natural defences) so that it attacks and kills the cancer cells.

To prepare this medicine, immune system cells called 'dendritic cells' are extracted from the patient's blood. The cells are then incubated (mixed) with a 'fusion protein', which is then absorbed into the cells. The fusion protein contains a fragment of mucin 1, a protein that is found in large amounts on the surface of cancer cells but is rarely found on normal cells. When the dendritic cells are injected back into the patient, the immune system is expected to recognise the mucin 1 as 'foreign'. This is expected to stimulate an immune response against mucin 1, resulting in the immune system attacking the cancerous cells in the body.

What is the stage of development of this medicine?

The effects of autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with ovarian cancer were ongoing.

At the time of submission, this 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 2 June 2010 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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	Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose	Treatment of ovarian cancer
Bulgarian	Автоложни дендритни клетки, пулсирани с рекомбинантен човешки-фюжън протеин (муцин 1 – глутатион С трансфераза), сдвоени с оксидирана полиманоза.	Лечение на рак на яйчниците
Czech	Autologní dendritické buňky kokultivované rekombinantním humáním fúzním proteinem(mucin 1 – glutathion S transferáza) spojená s oxidovanou polymannosou	Léčba karcinomu vaječníků
Danish	Autologe dendritceller tilsat rekombinant humant fusionsprotein (mucin 1 – glutathion s transferase) og koblet til oxideret polymannose.	Behandling af ovarie cancer
Dutch	Autologe dendritische cellen die met een geoxideerde polymannose gebonden recombinant humaan-fusie proteïne (mucin 1 – glutathione S transferase) behandeld werden	Behandeling van ovariumkanker
Estonian	Autoloogsed dendriidirakud koos rekombinantse inimese liitvalguga (mutsiin-1-glutatioon-S-transferraas), mis on seotud oksüdeeritud polümannoosiga.	Munasarjavähi ravi
Finnish	Autologisia dendriittisiä soluja, jotka ovat ladattu rekombinantilla humaanilla fuusioproteiniilla (musiini 1 – glutationi S transferaasi) yhdistettynä hapetettuun polymannoosiin	Munasarjasyövän hoito
French	Cellules dendritiques autologues pulsées par une protéine de fusion humaine recombinante (mucine 1 – transférase du glutathion S) couplée au polymannose oxide	Traitement du cancer de l'ovaire
German	Autologe dendritische Zellen, die mit an oxidierter Polymannose gebundenem rekombinanten humanen Fusionsprotein (Mucin-1-glutathione-S-Transferase) behandelt wurden	Behandlung des Ovarialkarzinoms
Greek	Αυτόλογα δενδριτικά κύτταρα επωασμένα με ανασυνδυασμένη ανθρώπινη χιμερική πρωτεΐνη (βλεννίνη 1 – γλουταθειόνη S τρανσφεράση) συνδεδεμένη με οξειδωμένη πολυμαννόζη.	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Oxidizált polymannóz-hoz kötött, rekombináns humán fúziós proteinnel (mucin 1 – glutation-transzferáz) kezelt autológ dendritsejtek	Petefészekrák kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	Cellule dendritiche autologhe pulsate con proteina umana ricombinante di fusione (mucin-1-glutatione-S-transferasi) accoppiata a polimannosio ossidato	Trattamento del carcinoma dell'ovaio
Latvian	Autologas dendrīt šūnas apstrādātas ar rekombinantu cilvēka izcelsmes saplūšanas proteīnu (micīna 1glutationa S transferāze) savienotas ar oksidētu polimannozi	Olnīcu vēža ārstēšanai
Lithuanian	Autologinės dendritinės ląstelės suplaktos su rekombinantiniu žmogaus sintezuotu baltymu (mucino 1 – glutationo S transferazė), sujungtos su oksiduota polimanoze	Kiaušidžių vėžio gydymas
Maltese	Ċelluli dendritiċi awtologużi pulsati ma' proteina ta' fużjoni umana rikombinanti (mucin 1 – glutathione S transferase) magħqudin ma' polymannose ossidat	Kura għal kanċer tal-ovarji
Polish	Autogenne komórki dendrytyczne wzbogacone rekombinowanym ludzkim białkiem fuzyjnym (połączenie mucyny 1 z transferazą S-glutationową) z przyłączonymi utlenionymi polimannozami.	Leczenie raka jajnika
Portuguese	Células dendríticas autólogas carregadas com a proteína de fusão recombinante (mucina 1 – glutationa S-transferase) humana acoplada a polimanase oxidada	Tratamento do carcinoma do ovário
Romanian	Celule dendritice autologe pulsate cu proteina de fuziune umană recombinantă (mucin-1 – glutation S transferază) cuplată cu polimanoză oxidată	Tratamentul cancerului ovarian
Slovak	Autológne dendritické bunky inkubované s rekombinantným ľudským fúznym proteínom (mucín 1 – glutatión S transferáza) spojeným s oxidovanou polymannózou	Liečba rakoviny vaječníkov
Slovenian	Avtologne dendritične celice spodbujene z rekombinantnim humanim fuzijskim proteinom (mucin 1 – glutation S transferaza), vezanim na oksidirano polimanozo	Zdravljenje raka na jajčnikih
Spanish	Células dendríticas autólogas cargadas con una proteína de fusión recombinante (mucina 1 glutation - S transferasa) combinada con polimanosa oxidada	Tratamiento del cáncer de ovario
Swedish	Autologa dendrittiska celler pulserade med rekombinant mänskligt fusionsprotein (mucin 1 – glutation-S-transferase) kopplat till oxiderad polymannos	Behandling av ovarialcancer

Language	Active ingredient	Indication
Norwegian	Autologe dendrittiske celler pulset med rekombinant humant fusjonsprotein (mucin 1 – glutation-S-transferase) koblet til oksidert polymannose	Behandling av eggstokkreft
Icelandic	Samgena griplu frumur púlsaðar með raðbrigða manna-samruna próteini (múcín 1 – glútatíón S transferasa tengt við oxideraðan pólýmannósa	Meðferð eggjastokkakrabbameins