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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous dendritic cells pulsed with allogeneic tumour cell lysate for the treatment of malignant mesothelioma

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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 16 January 2014, orphan designation (EU/3/13/1229) was granted by the European Commission to Amphera BV, the Netherlands, for autologous dendritic cells pulsed with allogeneic tumour cell lysate for the treatment of malignant mesothelioma.

What is malignant mesothelioma?

Malignant mesothelioma is a cancer that affects the mesothelial cells (found on the inner linings of the organs), mainly in the pleura (the lining of the lungs) and in the peritoneum (the lining of the abdominal cavity). It is usually caused by exposure to asbestos. Mesothelioma of the pleura causes difficulty breathing and chest pain, and mesothelioma of the peritoneum causes ascites (a build-up of fluid in the abdomen) and abdominal pain. Because the symptoms are not specific, the cancer is often detected at a late stage.

Malignant mesothelioma is life-threatening because it may lead to bowel obstruction, heart or breathing problems and lung infections. Patients have very poor survival, only living for a year, on average, after diagnosis.

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

At the time of designation, malignant mesothelioma affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 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 malignant mesothelioma was surgery followed by chemotherapy (medicines to treat cancer) or radiotherapy (treatment with radiation). If the disease was too advanced for surgery, chemotherapy alone was used. Only one medicine, pemetrexed, was specifically authorised in the EU for the treatment of malignant pleural mesothelioma.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with malignant mesothelioma because it works in a different way to current treatments and early preclinical studies suggested it might improve survival of patients with the condition. 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 activating the patient's immune system (the body's natural defences) so that it attacks and kills the cancer cells. The medicine is prepared from the patient's own (autologous) immune cells called dendritic cells. These cells are mixed in the laboratory with components of five different types of malignant mesothelioma cells. When the dendritic cells are injected back into the patient, it is expected that they will help the immune system recognise similar components in the patient's own malignant mesothelioma cells as 'foreign' and stimulate an immune response against them, helping to slow down or stop the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with malignant mesothelioma were ongoing.

At the time of submission, the medicinal product was not authorised anywhere in the EU for malignant mesothelioma 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 December 2013 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 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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 allogeneic tumour cell lysate	Treatment of malignant mesothelioma
Bulgarian	Автоложни дендритни клетки, натоварени с лизат от алогенни туморни клетки	Лечение на малигнен мезотелиом
Czech	Autologní dendritické buňky s lyzátem alogenní nádorových buněk	Léčba maligního mezoteliomu
Croatian	Autologne dendritičke stanice podražene staničnim lizatom alogenog tumora	Liječenje malignog mezotelioma
Danish	Autologe dendritiske celler pulsstimulerede med allogent lysat af tumorceller	Behandling af malignt mesotheliom
Dutch	Autologe dendritische cellen gepulseerd met allogeen tumorcellysaat	Behandeling van maligne mesothelioom
Estonian	Autoloogsed dendriitrakud, mis on laetud allogeense kasvajaraku lüsaadiga	Pahaloomulise mesotelioomi ravi
Finnish	Allogeenisellä kasvainsolulysaatilla ladatut autologiset dendriittisolut	Malignin mesotheliooman hoito
French	Cellules dendritiques autologues chargées en lysat cellulaire tumoral allogénique	Traitement du mésothéliome malin
German	Autologe dendritische Zellen, die mit allogenem Tumorzelllysat behandelt wurden	Behandlung des malignen Mesothelioms
Greek	Αυτόλογα δενδριτικά κύτταρα επεξεργασμένα με κυτταρόλυμα αλλογενή όγκου	Θεραπεία κακοήθους μεσοθηλιώματος
Hungarian	Allogén tumor sejtlizátum pulzált autológ dendritikus sejtek	Rosszindulatú mesothelioma kezelése
Italian	Cellule dendritiche autologhe indotte con lisato di cellule tumorali allogeniche	Trattamento del mesotelioma maligno
Latvian	Ar alogēnu audzēju šūnu lizātu pārslogotas autologas dendrītiskās šūnas	Ļaundabīgas mezoteliomas ārstēšana
Lithuanian	Autologinės dendritinės ląstelės pulsuojančios su alogeninių auglio ląstelių lizatu	Piktybinės mezoteliomos gydymas
Maltese	Ċelluli dendritiċi awtologużi pulsati ma' lysate ta' ċelluli tumurali alloġeniċi	Kura tal-mesoteljoma malinna
Polish	Autologiczne komórki dendrytowe obładowane lizatem allogenicznych komórek guza	Leczenie złośliwego międzybłoniaka
Portuguese	Células dendríticas autólogas activadas com lisado de células tumorais alogênicas	Tratamento do Mesotelioma maligno
Romanian	Celule dendriticeautologe încărcate cu lizat de celule tumorale alogene	Tratamentul mezoteliomului malign

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Autológne dendritické bunky pulzované lyzátom buniek alogénneho nádora	Liečba malígneho mezoteliómu
Slovenian	Avtologne dendritične celice, izpostavljene lizatualogenih tumorskih celic	Zdravljenje malignega mezotelioma
Spanish	Células dendríticas autólogas cargadas de lisado alogénico de células de tumor	Tratamiento del mesotelioma maligno
Swedish	Autologa dendritiska celler pulserade med allogent tumörscellysat	Behandling av malignt mesoteliom
Norwegian	Autologe dendritiske celler pulset med allogen svulst cellelysat	Behandling av malignt mesoteliom
Icelandic	Samgena griplufrumur púlseraðar með ósamgena æxlisfrumna lausn	Meðferð við illkynja miðþekjuæxli