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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine for the treatment of ovarian cancer

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in November 2010 on request of the sponsor.

On 28 October 2009, orphan designation (EU/3/09/682) was granted by the European Commission to MedImmune Ltd, United Kingdom, for anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine for the treatment of ovarian cancer.

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 started to spread to other parts of the body.

Ovarian cancer is a life-threatening disease that leads to poor long-term survival.

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 is equivalent to a total of around 151,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 504,800,000 (Eurostat 2009).

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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 the stage of the disease. 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 medicines and because early studies in experimental models indicate that it might improve the treatment of patients with this condition. These assumptions 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?

Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine is made up of two components:

- anti-EphA2 monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure (antigen) called EphA2. EphA2 is a receptor that is found in large amounts on the surface of cancer cells, such as the cells in ovarian cancer;
- maleimidocaproyl monomethylauristatin phenylalanine (mcMMAF), a substance that kills cancer cells by preventing cell division.

This medicine is expected to attach to ovarian cancer cells by binding to EphA2. Once the medicine is attached to these cells, it is expected to be taken inside the cells and release mcMMAF, which blocks cell division. This is expected to slow down the growth of ovarian cancer.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

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

At the time of submission, this medicine was not authorised anywhere in the EU for ovarian cancer. Orphan designation for this product had been granted in the United States of America for ovarian cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 September 2009 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Anti-EphA2 monoclonal antibody conjugated to maleimidocaproyl monomethylauristatin phenylalanine	Treatment of ovarian cancer
Bulgarian	Анти-EphA2 моноклонално антитяло, конюгирано с малеимидокапроил-монометилауристатин-фенилаланин	Лечение на рак на яйчниците
Czech	Monoklonální protilátka Anti-EphA2 konjugovaná s maleimidocaproyl monometylauristatin fenylalaninem	Léčba karcinomu vaječníků
Danish	Monoklonalt antistof mod EphA2, konjugeret til maleimidocaprylmonomethylauristatinphenylalanin	Behandling af ovarie cancer
Dutch	Anti-EphA2 monoklonaal antilichaam geconjugeerd aan maleimidocaproyl monomethylauristatine fenylalanine	Behandeling van ovariumkanker
Estonian	Maleimidokaproüül monometüülauristatiin-fenüülalaniiniga konjugeeritud anti-EphA2 monoklonaalne antikeha	Munasarjavähi ravi
Finnish	Maleimidikaproiini-monometyyliauristatiini-fenyylialaniiniin konjugoitunut monoklonaalinen anti-EphA2-vasta-aine	Munasarjasyövän hoito
French	Anticorps monoclonal anti-EphA2 conjugué à la phénylalanine monométhylauristatine de maleimido-caproyl	Traitement du cancer de l'ovaire
German	Anti-EphA2 monoklonaler Antikörper konjugiert mit Maleimidocaproyl-Monomethylauristatin-Phenylalanin	Behandlung des Ovarialkarzinoms
Greek	Μονοκλωνικό αντίσωμα αντι-EphA2 συζευγμένο σε φαινυλαλανίνη μηλεϊμιδοκαπροϋλο μονομεθυλο αουριστατίνης	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Maleimidokaproil-monometilauristatin-fenilalaninnal konjugált anti-EphA2 monoklonális antitest	Petefészekrák kezelése
Italian	Anticorpo monoclonale anti-EphA2 coniugato a maleimidocaproil, monometilauristatin-fenilalanina	Trattamento del carcinoma dell'ovaio
Latvian	anti-EphA2 monoklonālas antivielas, kas saistītas ar maleimidokaproilmonometilauristatīna fenilalanīnu	Olnīcu vēža ārstēšanai
Lithuanian	Anti-EphA2 monokloninis antikūnas, konjuguotas su maleimidokaproilo monometilauristatino fenilalaninu	Kiaušidžių vėžio gydymas
Maltese	Antikorp monoklonali anti-EphA2 ikkonjugat ma' maleimidocaproyl monomethylauristatin phenylalanine	Kura tal-kanċer ta' l-ovarji
Polish	Przeciwciało monoklonalne przeciw EphA2 sprzężone z maleimidokaproilo-MMAF (mcMMAF)	Leczenie raka jajnika
Portuguese	Anticorpo monoclonal anti-EphA2 conjugado com fenilalanina monometilauristatina maleimidocaproil	Tratamento do carcinoma do ovário
Romanian	Anticorp monoclonal Anti-EphA2 conjugat cu maleimidocaproil monometilauristatin fenilalanină	Tratamentul cancerului ovarian

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Monoklonálna protilátka Anti-EphA2 konjugovaná s maleimidokaproyl monometylauristatín fenylalanínom	Liečba rakoviny vaječníkov
Slovenian	Monoklonsko protitelo proti EphA2, konjugirano na maleimidokaproilmonometilauristatinfenilalanin	Zdravljenje raka na jajčnikih
Spanish	Anticuerpo monoclonal anti-EphA2 conjugado con maleimidocaproil monometilauristatina-fenilalanina	Tratamiento del cáncer de ovario
Swedish	Anti-EphA2 monoklonal antikropp konjugerad till maleimidocaproyl-monometylauristatin-fenylalanin	Behandling av ovarialcancer
Norwegian	Monoklonalt anti-EphA2-antistoff konjugert til maleimidokapryl monometylauristatin fenylalanin	Behandling av eggstokkreft
Icelandic	Einstofna mótefni gegn EphA2 samtengt við maleímídocapróýl mónómethýlauristatín phenýlalanín	Meðferð eggjastokkakrabbameins