6 May 2015
EMA/COMP/118047/2015
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4 for the treatment of ovarian cancer

On 19 March 2015, orphan designation (EU/3/15/1458) was granted by the European Commission to ImmunoGen Europe Limited, United Kingdom, for humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4 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 not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 154,000 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 showed that it may improve the outcome

*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 512,900,000 (Eurostat 2015).

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of patients whose disease did not respond or had 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?

This medicine is made up of two active components, which are linked together:

- a monoclonal antibody (a type of protein) that has been designed to recognise and attach to certain receptors called folate receptors 1, which are present in high amounts on certain cancer cells including some ovarian cancer cells.
- maytansinoid DM4, a toxic substance that kills cells when they attempt to divide and grow. DM4 attaches to a protein in cells called 'tubulin', which is important in the formation of the internal 'skeleton' that cells need to assemble when they divide. By attaching to tubulin in cancer cells, DM4 stops the formation of this skeleton, preventing the division and growth of the cancer cells.

The medicine is expected to attach to the folate receptor of the cancer cell and enter the cell. The medicine is then expected to release the maytansinoid DM4 component inside the cell, causing it to die.

## 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 of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 February 2015recommending the granting of this designation.

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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 | Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4 | Treatment of ovarian cancer |
| Bulgarian | Хуманизирано моноклонално антитяло към антифолатен рецептор 1, конюгирано с майтансиноид DM4 | Лечение на рак на яйчниците |
| Croatian | Humanizirano monoklonsko protutijelo konjugirano s majtanzinoidom DM4 usmjereno protiv folatnog receptora 1 | Liječenje raka jajnika |
| Czech | Humanizovaná monoklonální protilátka specifická proti anti-folátovému receptoru 1 konjugovaná s maytansinoidem DM4 | Léčba karcinomu vaječníků |
| Danish | Humaniseret antifolatreceptor 1 monoklonalt antistof konjugeret til maytansinoid DM4 | Behandling af ovarie cancer |
| Dutch | Gehumaniseerd monoklonaal antilichaam tegen folaatreceptor-1, geconjugeerd met maytansinoïd-DM4 | Behandeling van ovariumkanker |
| Estonian | Humaniseeritud anti-folaadi retseptor 1 monoklonaalne antikeha konjugeeritud maitansinoid DM4 | Munasarjavähi ravi |
| Finnish | Humanisoidun antifolaattireseptori 1:n monoklonaalinen vasta-aine konjugoituna maytansinoidi DM4:ään | Munasarjasyövän hoito |
| French | Anticorps monoclonal anti-récepteur 1 de folate humanisé conjugué au maytansinoïde DM4 | Traitement du cancer de l'ovaire |
| German | Maytansinoid DM4 konjugierter, humanisierter monoklonaler Antikörper gegen Folatrezeptor 1 | Behandlung des Ovarialkarzinoms |
| Greek | Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι υποδοχέα του φυλλικού οξέος 1 συζευγμένο με μεϋτανσινοειδές DM4 | Θεραπεία του καρκίνου των ωοθηκών |
| Hungarian | Maytansinoid DM4-hez konjugált humanizált anti-folát receptor 1 | Petefészekrák kezelése |
| Italian | Anticorpo monoclonale umanizzato anti-recettore 1 del folato coniugato al maitansinoide DM4 | Trattamento del carcinoma dell'ovaio |
| Latvian | Humanizēta monoklonālā antiviela pret folāta receptoru 1, kas konjugēta ar maitansinoīdu DM4 | Olnīcu vēža ārstēšana |
| Lithuanian | Humanizuotas anti-folato receptoriaus 1 monokloninis antikūnas, konjuguotas su maitanzinoidu DM4 | Kiaušidžių vėžio gydymas |
| Maltese | Antikorp monoklonali għal riċettur 1 kontra l-folate umanizzat ikkonjugat ma' maytansinoid DM44 | Kura tal-kanċer ta' l-ovarji |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Polish | Humanizowane przeciwciało monoklonalne przeciw receptorowi folianu 1 sprzężone z majtanzynoidem DM4 | Leczenie raka jajnika |
| Portuguese | Anticorpo monoclonal humanizado anti-recetor 1 de folato conjugado com DM4 maitansinóide | Tratamento do carcinoma do ovário |
| Romanian | Anticorp monoclonal umanizat anti-receptor 1 pentru folat, conjugat cu maitansinoid DM4 | Tratamentul cancerului ovarian |
| Slovak | Humanizovaná monoklonálna protilátka špecifická proti anti-folátovému receptoru 1 konjugovaná s maytanzinoidom DM4 | Liečba rakoviny vaječníkov |
| Slovenian | Z majtanzinoidom DM4 konjugirano humanizirano antifolatno monoklonsko protitelo usmerjeno proti receptorju 1 | Zdravljenje raka na jajčnikih |
| Spanish | Anticuerpo monoclonal humanizado contra el receptor 1 del folato conjugado con maitansinoide DM4 | Tratamiento del cáncer de ovario |
| Swedish | Humaniserad antifolat-receptor 1 monoklonal antikropp konjugerad med maytansinoid-DM4 | Behandling av ovarialcancer |
| Norwegian | Humanisert anti-folat reseptor 1 monoklonalt anstistoff konjugert til maytansinoid DM4 | Behandling av eggstokkreft |
| Icelandic | Mannaðlagað andfólat viðtaka 1 einstofna mótefni tengt maytansínóíð DM4 | Meðferð eggjastokkakrabbameins |