



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fosbretabulin tromethamine for the treatment of ovarian cancer

On 17 August 2013, orphan designation (EU/3/13/1154) was granted by the European Commission to Diamond BioPharm Limited, United Kingdom, for fosbretabulin tromethamine 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 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 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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 fosbretabulin tromethamine might be of significant benefit for patients with ovarian cancer because studies suggest it might improve the treatment of ovarian cancers resistant to platinum treatments, when used in combination with the

*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 509,000,000 (Eurostat 2013).



chemotherapy medicines carboplatin and paclitaxel. 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?

Fosbretabulin tromethamine is a medicine that targets the cells of blood vessels supplying the tumour, disrupting small structures called microtubules within the cells which allow these cells to divide. By targeting these blood vessels, it is expected to cause the death of tumour cells by cutting off their blood supply, and thereby slow down the progression of the disease.

Fosbretabulin tromethamine is expected to be given in combination with other medicines (carboplatin and paclitaxel) that damage the cancer cells in different ways.

What is the stage of development of this medicine?

The effects of fosbretabulin tromethamine have been evaluated in experimental models.

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

At the time of submission, fosbretabulin tromethamine was not authorised anywhere in the EU for ovarian cancer. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 June 2013 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Fosbretabulin tromethamine	Treatment of ovarian cancer
Bulgarian	Фосбретабулин трометамин	Лечение на рак на яйчниците
Czech	Fosbretabulin trometamin	Léčba karcinomu vaječníků
Croatian	Fosbretabulinov trometamin	Liječenje raka jajnika
Danish	fosbretabulin-trometamin	Behandling af ovarie cancer
Dutch	Fosbretabuline tromethamine	Behandeling van ovariumkanker
Estonian	Fosbretabuliin trometamiin	Munasarjavähi ravi
Finnish	Fosbretabuliini-trometamiini	Munasarjasyövän hoito
French	Trométhamine fosbretabuline	Traitement du cancer de l'ovaire
German	Fosbretabulin-Tromethamin	Behandlung des Ovarialkarzinoms
Greek	Φοσβρεταβουλίνη τρομεθαμίνη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	oszbretabulin trometamin	Petefészekrák kezelése
Italian	Fosbretabulina trometamina	Trattamento del carcinoma dell'ovaio
Latvian	Fosbretabulīna trometamīns	Olnīcu vēža ārstēšana
Lithuanian	Fosbretabulino trometaminas	Kiaušidžių vėžio gydymas
Maltese	Fosbretabulin tromethamine	Kura tal-kanċer ta' l-ovarji
Polish	Fosbretabuliny trometamina	Leczenie raka jajnika
Portuguese	Trometamina de fosbretabulina	Tratamento do carcinoma do ovário
Romanian	Fosbretabulin trometamină	Tratamentul cancerului ovarian
Slovak	Fosbretabulín trometamín	Liečba rakoviny vaječníkov
Slovenian	Fosbretabulinov trometamin	Zdravljenje raka na jajčnikih
Spanish	Fosbretabulina trometamol	Tratamiento del cáncer de ovario
Swedish	Fosbretabulintrometamin	Behandling av ovarialcancer
Norwegian	Fosbretabulintrometamol	Behandling av eggstokkreft
Icelandic	Fosbretabúlín trómetamín	Meðferð eggjastokkkrabbameins

¹ At the time of designation