



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Veliparib for the treatment of ovarian cancer

On 17 December 2010, orphan designation (EU/3/10/830) was granted by the European Commission to Abbott Laboratories, United Kingdom, for veliparib for the treatment of ovarian cancer.

The sponsorship was transferred to AbbVie Ltd, United Kingdom, in January 2013.

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 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 2.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 106,000 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 veliparib might be of significant benefit for patients with ovarian cancer because it works in a different way to existing treatments, and early studies indicate that it might be used in combination with existing treatment to 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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).



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?

This medicine is expected to work by blocking some enzymes known as poly(ADP-ribose) polymerase (PARP)-1 and -2. These enzymes are used to repair damaged DNA. By blocking PARP-1 and -2, the medicine prevents cancer cells from repairing their DNA, eventually killing them while allowing ordinary cells to survive. It is expected that the medicine will be able to be used on its own to kill those cancer cells that do not have other ways of repairing DNA damage, such as those with abnormalities in the BRCA-1 or BRCA-2 genes, which are found in some ovarian cancer patients. The medicine is also expected to be used in combination with other cytotoxic (cell-killing) cancer medicines that cause DNA damage, such as chemotherapy, where it is expected to enhance their activity by preventing DNA repair.

What is the stage of development of this medicine?

The effects of veliparib have been evaluated in experimental models.

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

At the time of submission, veliparib was not authorised anywhere in the EU for the treatment of ovarian cancer. Orphan designation of veliparib had been granted in the United States of America for the treatment of epithelial ovarian cancer in combination with DNA-damaging agents.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2010 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	Veliparib	Treatment of ovarian cancer
Bulgarian	Велипариб	Лечение на рак на яйчниците
Czech	Veliparib	Léčba karcinomu vaječníků
Danish	Veliparib	Behandling af ovarie cancer
Dutch	Veliparib	Behandeling van ovariumkanker
Estonian	Veliparib	Munasarjavähi ravi
Finnish	Veliparibi	Munasarjasyövän hoito
French	Veliparib	Traitement du cancer de l'ovaire
German	Veliparib	Behandlung des Ovarialkarzinoms
Greek	Βελιπαρίμπη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Veliparib	Petefészekrák kezelése
Italian	Veliparib	Trattamento del carcinoma dell'ovaio
Latvian	Veliparibs	Olnīcu vēža ārstēšanai
Lithuanian	Veliparibas	Kiaušidžių vėžio gydymas
Maltese	Veliparib	Kura għal kanċer tal-ovarji
Polish	Weliparyb	Leczenie raka jajnika
Portuguese	Veliparib	Tratamento do carcinoma do ovário
Romanian	Veliparib	Tratamentul cancerului ovarian
Slovak	Veliparib	Liečba rakoviny vaječníkov
Slovenian	Veliparib	Zdravljenje raka na jajčnikih
Spanish	Veliparib	Tratamiento del cáncer de ovario
Swedish	Veliparib	Behandling av ovarialcancer
Norwegian	Veliparib	Behandling av eggstokkrefte
Icelandic	Veliparíð	Meðferð eggjastokkkrabbameins

¹ At the time of designation