30 January 2015
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Epothilone B for the treatment of ovarian cancer

First publication	6 January 2003
Rev.1: administrative update	8 January 2003
Rev.2: administrative update	11 June 2003
Rev.3: withdrawal from the Community Register	23 March 2011
Rev.4: sponsor's change of address	30 January 2015
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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2011 on request of the Sponsor.

On 22 March 2002, orphan designation (EU/3/02/098) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for epothilone B for the treatment of ovarian cancer.

What is ovarian cancer?

Tumours that begin in the ovaries are known as ovarian tumours. Tumours which have potential to grow rapidly and infiltrate surrounding healthy tissues are called ovarian cancers. Due to the absence of symptoms in early stages of the disease the majority of the patients are diagnosed when the tumours have spread locally or to distant parts of the body. Ovarian cancer is a life-threatening condition.

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

At the time of designation, ovarian cancer affected approximately 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 87,000 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?

Several anticancer medicinal products were authorised for the condition in the Community at the time of submission of the application for orphan designation. Although a significant percentage of patients respond to the initial chemotherapy, most patients relapse and respond moderately or poorly to subsequent chemotherapy. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that epothilone B might be of potential significant benefit for the treatment of ovarian cancer, particularly based on efficacy demonstrated in experimental models containing tumour cells resistant to other anticancer medicinal products.

How is this medicine expected to work?

Epothilone B is the first representative of a new class of natural compounds, which like another group of cytotoxic cancer drugs, the taxanes (docetaxel and paclitaxel), promotes polymerisation of tubulin heterodimers into microtubules and stabilises preformed microtubules. The outcome is reduction of free tubulin in the cell, which results in inhibition of cell division and tumour growth.

What is the stage of development of this medicine?

At the time of the orphan drug designation application epothilone B had been tested in experimental models and in two early clinical studies in patients with different tumours.

No clinical trials in patients with ovarian cancer had been initiated, at the time of submission of the application for orphan designation.

Epothilone B had not been marketed anywhere worldwide for ovarian cancer or designated as an orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 23 January 2002 recommending 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.

[*]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.
At the time of designation, this represented a population of 380,600,000 (Eurostat 2002).

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	Epothilone B	Treatment of ovarian cancer
Danish	Epothilon B	Behandling af cancer ovarii
Dutch	Epothilon B	Voor het behandelen van ovarium kanker
Finnish	Epotiloni B	Munasarjasyövän hoito
French	Epothilone B	Traitement du cancer ovarien
German	Epothilon B	Behandlung des Ovarialkarzinoms
Greek	Epothilone B	Αγωγή στον καρκίνο της ωοθήκης
Italian	Epotilone B	Trattamento del cancro dell'ovaio
Portuguese	Epotilona B	Tratamento do cancro do ovário
Spanish	Epotilona B	Tratamiento de cancer de ovario
Swedish	Epotilon B	Behandling av ovarialcancer

[1] At the time of designation