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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

trabectedin for the treatment of ovarian cancer

On 17 October 2003, orphan designation (EU/3/03/171) was granted by the European Commission to PharmaMar S.A., Spain, for trabectedin 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.

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

At the time of designation, ovarian cancer affected approximately 2.4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 92,000 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 (using drugs to kill cancer cells), most ovarian cancers grow again and respond moderately or poorly to subsequent chemotherapy.

Trabectedin might be of potential significant benefit for the treatment of ovarian cancer, because it may offer a new mechanism of action to fight the disease. This assumption will have to be confirmed. This will be necessary to maintain the orphan status.

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

How is this medicine expected to work?

Trabectedin is a substance isolated from a small marine animal called sea squirt living on mangrove roots in the Caribbean Sea. Trabectedin could exert antitumour activity via several mechanisms, such as bending DNA (a part of the fundamental genetic material) in the cells and thereby making it difficult for the cell to multiply. Furthermore, trabectedin might induce a decrease in the speed of cell cycle multiplication and eventually a cell cycle arrest, thereby preventing cancer cells to grow or at least slowing down the growth of cancer cells.

What is the stage of development of this medicine?

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 trabectedin was not marketed anywhere worldwide for ovarian cancer or designated as orphan medicinal product elsewhere for this condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 September 2003 a positive opinion recommending the grant of the above-mentioned designation.

Update: trabectedin (Yondelis) has been authorised in the EU since 28 October 2009. Yondelis in combination with pegylated liposomal doxorubicin (PLD) is indicated for the treatment of patients with relapsed platinum-sensitive ovarian cancer

For more information on Yondelis, see: http://www.ema.europa.eu/htms/human/epar/y.htm

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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 European Union) 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:

PharmaMar S.A.
Polígono Industrial La Mina
Av. de los Reyes 1
28770 Colmenar Viejo
Madrid
Spain
Telephone: (34-91) 846 60 00
Telefax: (34-91) 823 45 03
E-mail: pharmamar@pharmamar.com

Patient associations' contact points

CancerBACUP
3 Bath Place, Rivington Street
London EC2A 3JR
United Kingdom
Tel: +44 (0)20 7696 9003

Ligue Nationale Contre le Cancer
14 rue Corvisart
F-75013 Paris
France

Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Trabectedin	Treatment of ovarian cancer
Danish	Trabectedin	Behandling af ovarie cancer
Dutch	Trabectedine	Behandeling van ovariumkanker
Finnish	Trabektediini	Munasarjasyövän hoito
French	Trabectédine	Traitement du cancer de l'ovaire
German	Trabectedin	Behandlung von Ovarialkarzinom
Greek	Τραβεκτεδίνη	Θεραπεία του καρκίνου των ωοθηκών
Italian	Trabectedina	Trattamento del carcinoma dell'ovaio
Portuguese	Trabectedina	Tratamento do cancro do ovário
Spanish	Trabectedina	Tratamiento del cáncer de ovario
Swedish	Trabectedin	Behandling av ovarialcancer