Public summary of opinion on orphan designation

Lurbinectedin for the treatment of ovarian cancer

On 10 October 2012, orphan designation (EU/3/12/1053) was granted by the European Commission to Pharma Mar SA Sociedad Unipersonal, Spain, for lurbinectedin for the treatment of ovarian cancer.

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 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 approximately 2.8 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 142,000 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 lurbinectedin might be of significant benefit for patients with ovarian cancer because early studies in experimental model show that it might slow down the growth of the cancer when used on its own. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

[*]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 506,300,000 (Eurostat 2011).

An agency of the European Union

How is this medicine expected to work?

Lurbinectedin is anticancer substance that belongs to the group 'alkaloids'. It is expected to kill cancer cells by attaching to their DNA, thereby preventing the cell from making more copies of DNA and making proteins. As a result, cancer cells cannot reproduce and this slows down the growth of the cancer.

What is the stage of development of this medicine?

The effects of lurbinectedin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with lurbinectedin in patients with ovarian cancer was ongoing.

At the time of submission, lurbinectedin was not authorised anywhere in the EU for ovarian cancer or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 September 2012 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.

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	Lurbinectedin	Treatment of ovarian cancer
Bulgarian	лурбинектедин	Лечение на рак на яйчниците
Czech	Lurbinektedin	Léčba karcinomu vaječníků
Danish	Lurbinectedin	Behandling af ovarie cancer
Dutch	Lurbinectedine	Behandeling van ovariumkanker
Estonian	Lurbinektediin	Munasarjavähi ravi
Finnish	Lurbinektediini	Munasarjasyövän hoito
French	Lurbinectédine	Traitement du cancer de l'ovaire
German	Lurbinektedin	Behandlung des Ovarialkarzinoms
Greek	λουρμπινεκτεδίνη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Lurbinectedin	Petefészekrák kezelése
Italian	Lurbinectedina	Trattamento del carcinoma dell'ovaio
Latvian	Lurbinektedīns	Olnīcu vēža ārstēšana
Lithuanian	Lurbinektedinas	Kiaušidžių vėžio gydymas
Maltese	Lurbinectedin	Kura tal-kanċer ta' l-ovarji
Polish	Lurbinektedyna	Leczenie raka jajnika
Portuguese	Lurbinectedina	Tratamento do carcinoma do ovário
Romanian	Lurbinectedină	Tratamentul cancerului ovarian
Slovak	Lurbinektedín	Liečba rakoviny vaječníkov
Slovenian	Lurbinektedin	Zdravljenje raka na jajčnikih
Spanish	Lurbinectedina	Tratamiento del cáncer de ovario
Swedish	Lurbinektedin	Behandling av ovarialcancer
Norwegian	Lurbinektedin	Behandling av eggstokkreft
Icelandic	Lúrbínektedín	Meðferð eggjastokkakrabbameins

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[1] At the time of designation