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EMA/COMP/613865/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rucaparib for the treatment of ovarian cancer

On 10 October 2012, orphan designation (EU/3/12/1049) was granted by the European Commission to Clovis Oncology UK Limited, United Kingdom, for rucaparib 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 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 not more than 2.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of not more than 106,000 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, particularly platinum-based medicines).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for some patients with ovarian cancer, based on early data showing clinical response in ovarian cancer patients who have a mutation in a gene called 'BRCA' and those whose cancers are resistant to

[*]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).

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platinum-based treatments. 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?

Rucaparib is expected to work by blocking the activity of a family of proteins called poly(ADP-ribose) polymerases (PARPs). These proteins play an important role in the repair of DNA in cells. Some cancer cells, including those with a mutation in the BRCA gene, are particularly dependent on PARPs to repair damaged DNA because they lack other proteins for repairing DNA that normal cells have.

By blocking the activity of PARPs in cancer cells, rucaparib is expected to stop the cancer cells from being able to repair damaged DNA and this eventually leads to the death of the cancer cells, thereby slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of rucaparib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, preliminary clinical trials with rucaparib including patients with ovarian cancer were ongoing.

At the time of submission, rucaparib was not authorised anywhere in the EU for ovarian cancer. Orphan designation of rucaparib has been granted in the United States of America 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	Rucaparib	Treatment of ovarian cancer
Bulgarian	Рукапариб (Rucaparib)	Лечение на рак на яйчниците
Czech	Rucaparib	Léčba karcinomu vaječníků
Danish	Rucaparib	Behandling af ovarie cancer
Dutch	Rucaparib	Behandeling van ovariumkanker
Estonian	Rucaparib	Munasarjavähi ravi
Finnish	Rukaparibi	Munasarjasyövän hoito
French	Rucaparib	Traitement du cancer de l'ovaire
German	Rucaparib	Behandlung des Ovarialkarzinoms
Greek	Ρουκαπαρίμπη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Rucaparib	Petefészekrák kezelése
Italian	Rucaparib	Trattamento del carcinoma dell'ovaio
Latvian	Rukaparibs	Olnīcu vēža ārstēšanai
Lithuanian	Rukaparibas	Kiaušidžių vėžio gydymas
Maltese	Rucaparib	Kura għal kanċer tal-ovarji
Polish	Rukaparib	Leczenie raka jajnika
Portuguese	Rucaparib	Tratamento do carcinoma do ovário
Romanian	Rucaparib	Tratamentul cancerului ovarian
Slovak	Rukaparib	Liečba rakoviny vaječníkov
Slovenian	Rukaparib	Zdravljenje raka na jajčnikih
Spanish	Rucaparib	Tratamiento del cáncer de ovario
Swedish	Rucaparib	Behandling av ovarialcancer
Norwegian	Rukaparib	Behandling av eggstokkreft
Icelandic	Rúcaparíb	Meðferð eggjastokkakrabbameins

[1] At the time of designation