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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Alisertib for the treatment of ovarian cancer

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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.	

On 8 November 2012, orphan designation (EU/3/12/1064) was granted by the European Commission to Takeda Global Research and Development Centre Ltd, United Kingdom, for alisertib for the treatment of ovarian cancer.

In November 2013, Takeda Global Research and Development Centre (Europe) Ltd changed name to Takeda Development Centre Europe Ltd.

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 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 153,000 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).

*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 509,000,000 (Eurostat 2012).



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 with platinum-based medicines.

The sponsor has provided sufficient information to show that alisertib might be of significant benefit for patients. Early studies showed improved anticancer activity when used in combination with currently authorised medicines, and a favourable survival rate has been seen in a small group of patients with ovarian cancer that was resistant or stopped responding to treatment with platinum-based medicines. 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?

Alisertib is an Aurora A kinase inhibitor. This means that it works by blocking the activity of an enzyme known as Aurora A kinase, which plays an important role in the control of cell division. Aurora A kinase is found in high amounts in cancer cells. By blocking this enzyme, the medicine is expected to kill dividing cancer cells, thereby inhibiting or slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of alisertib have been evaluated in experimental models.

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

At the time of submission, alisertib was not authorised anywhere in the EU for ovarian cancer. Orphan designation of alisertib had 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 October 2012 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 substance	Indication
English	Alisertib	Treatment of ovarian cancer
Bulgarian	Алисертиб	Лечение на рак на яйчниците
Czech	Aliserti	Léčba karcinomu vaječníků
Danish	Alisertib-	Behandling af ovarie cancer
Dutch	Alisertib	Behandeling van ovariumkanker
Estonian	Alisertiib	Munasarjavähi ravi
Finnish	Alisertibi	Munasarjasyövän hoito
French	Alisertib	Traitement du cancer de l'ovaire
German	Alisertib	Behandlung des Ovarialkarzinoms
Greek	Αλίσερτιμπη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Aliszertib-	Petefészekrák kezelése
Italian	Alisertib	Trattamento del carcinoma dell'ovaio
Latvian	Alisertibs	Olnīcu vēža ārstēšanai
Lithuanian	Alisertibas	Kiaušidžių vėžio gydymas
Maltese	Alisertib	Kura tal kanċer tal-ovarji
Polish	Alisertyb	Leczenie raka jajnika
Portuguese	Alisertibe	Tratamento do carcinoma do ovário
Romanian	Alisertib	Tratamentul cancerului ovarian
Slovak	Alisertib	Liečba rakoviny vaječníkov
Slovenian	Alisertib	Zdravljenje raka na jajčnikih
Spanish	Alisertib	Tratamiento del cáncer de ovario
Swedish	Alisertib	Behandling av ovarialcancer
Norwegian	Alisertib	Behandling av eggstokkreft
Icelandic	Alisertib	Meðferð eggjastokkakrabbameins

¹ At the time of designation