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Public summary of opinion on orphan designation

Ofranergene obadenovec for the treatment of ovarian cancer

On 16 October 2017, orphan designation (EU/3/17/1926) was granted by the European Commission to Envigo Pharma Consulting Limited, United Kingdom, for ofranergene obadenovec (also known as VB-111) for the treatment of ovarian cancer.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries, the two organs in the female reproductive system that produce eggs. Most ovarian cancers occur in women aged over 50 years. Due to the absence of clear symptoms in the early stages of the disease, the majority of women are diagnosed when the cancer has spread to other parts of the body.

Ovarian cancer is a 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 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 154,000 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 this medicine might be of significant benefit for patients with ovarian cancer because results from early studies suggest that, when used in patients in whom platinum-based treatments have failed, the medicine may increase the length of time patients live compared with other authorised treatments.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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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?

Ovarian cancer, like many solid tumours, relies on the growth of new blood vessels (angiogenesis) to obtain the nutrients that cancer cells need to grow and spread.

This medicine is made up of a virus that contains the gene for an engineered protein made up from two parts called Fas and TNFR1. The medicine has been designed so that the engineered protein is produced only in cells that line up the new blood vessels that supply the cancer tissue. When a substance, TNF, which is produced by the body, attaches to the TNFR1 part of the protein, it activates the Fas part which is involved in the regulation of cell death (apoptosis). This is expected to cause the death of the blood vessel cells, slowing down the growth of the cancer.

The type of virus used in this medicine ('adenovirus') is modified so that it does not cause disease in humans.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for ovarian cancer or designated as an orphan medicinal product elsewhere for this condition. Orphan designation of the medicine had been granted in the EU for the treatment of glioma (a type of brain cancer).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Ofranergene obadenovec	Treatment of ovarian cancer
Bulgarian	Офранерген обаденовековadenovec	Лечение на рак на яйчниците
Croatian	Ofranergen obadenovec	Liječenje raka jajnika
Czech	Ofranergen obadenovec	Léčba karcinomu vaječníků
Danish	Ofranergene obadenovec	Behandling af ovarie cancer
Dutch	Ofranergene obadenovec	Behandeling van ovariumkanker
Estonian	Ofranergene obadenovec	Munasarjavähi ravi
Finnish	Ofranergene obadenovec	Munasarjasyövän hoito
French	Ofranergène obadénovec	Traitement du cancer de l'ovaire
German	Ofranergene obadenovec	Behandlung des Ovarialkarzinoms
Greek	Ofranergene obadenovec	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Ofranergén obadenovek	Petefészekrák kezelése
Italian	Ofranergene obadenovec	Trattamento del carcinoma dell'ovaio
Latvian	Ofranergēna obadenoveks	Olnīcu vēža ārstēšana
Lithuanian	Ofranergenas obadenovekas	Kiaušidžių vėžio gydymas
Maltese	Ofranergene obadenovec	Kura tal-kanċer ta' l-ovarji
Polish	Ofranergen obadenowek	Leczenie raka jajnika
Portuguese	Ofranergene obadenovec	Tratamento do carcinoma do ovário
Romanian	Ofranergen obadenovec	Tratamentul cancerului ovarian
Slovak	Ofranergene obadenovec	Liečba rakoviny vaječníkov
Slovenian	Ofranergen obadenovec	Zdravljenje raka na jajčnikih
Spanish	Ofranergén obadenovec	Tratamiento del cáncer de ovario
Swedish	Ofranergene obadenovec	Behandling av ovarialcancer
Norwegian	Ofranergene obadenovec	Behandling av eggstokkreft
Icelandic	Ófranergen óbadenóvek	Meðferð eggjastokkakrabbameins

[1] At the time of designation