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Public summary of opinion on orphan designation

DNA plasmid encoding IL-12 p35 and p40 genes for the treatment of ovarian cancer

On 22 April 2020, orphan designation EU/3/20/2266 was granted by the European Commission to FGK Representative Service GmbH, Germany, for DNA plasmid encoding IL-12 p35 and p40 genes (also known as GEN-1) 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, it is usually 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 4.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 254,000 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 the medicine might be of significant benefit for patients with ovarian cancer because early results suggest that the medicine used together

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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with authorised treatments provides greater benefits to patients whose cancer had come back or as initial treatment before surgery than the authorised treatments alone.

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?

The medicine contains 2 genes responsible for making a protein called interleukin-12 (IL-12) which stimulates the immune system (the body's natural defences). When injected locally, next to the ovarian cancer, the genes are expected to produce IL-12 which will in turn make the immune system more active and encourage it to attack the cancer cells. This should help reduce the size of the cancer.

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 the treatment of ovarian cancer. Orphan designation of the medicine had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 19 March 2020, 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.

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	DNA plasmid encoding IL-12 p35 and p40 genes	Treatment of ovarian cancer
Bulgarian	ДНК плазмид кодиращ гени IL-12 p35 и p40	Лечение на рак на яйчниците
Croatian	Plazmidni DNK koji kodira gene IL-12 p35 i p40	Liječenje raka jajnika
Czech	DNA plazmid kódující geny p35 a p40 IL-12	Léčba karcinomu vaječníků
Danish	DNA-plasmid kodende for IL 12 p35 og p40 gener	Behandling af ovarie cancer
Dutch	DNA plasmide dat codeert voor het IL-12p35 en p40 gen	Behandeling van ovariumkanker
Estonian	IL-12 alaühikute p35 ja p40 geene kodeeriv DNA plasmiid	Munasarjavähi ravi
Finnish	IL-12 p35- ja p40-geenejä koodaava plasmidi-DNA	Munasarjasyövän hoito
French	Plasmide d'ADN portant les gènes codant pour les sous unités p35 et p40 de l'interleukine 12 (IL 12)	Traitement du cancer de l'ovaire
German	DNA-Plasmid, das IL-12 p35- und p40-Gene kodiert	Behandlung des Ovarialkarzinoms
Greek	Πλασμίδιο DNA που κωδικοποιεί τα γονίδια p35 και p40 της IL-12	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Az IL-12 p35 és a p40 gént kódoló DNS plazmid	Petefészekrák kezelése
Italian	Plasmide di DNA codificante i geni p35 e p40 dell'IL-12	Trattamento del carcinoma dell'ovaio
Latvian	IL 12 p35 un p40 gēnus kodējoša DNS plazmīda	Olnīcu vēža ārstēšana
Lithuanian	DNR plazmidė, koduojanti IL 12 p35 ir p40 genus	Kiaušidžių vėžio gydymas
Maltese	Plażmid tad-DNA li jikkodifika l-ġeni IL-12 p35 u p40	Kura tal-kanċer ta' l-ovarji
Polish	Plazmid DNA kodujący geny IL-12 p35 i p40	Leczenie raka jajnika
Portuguese	Plasmídeo de ADN que codifica os genes p35 e p40 da IL-12	Tratamento do carcinoma do ovário
Romanian	Plasmidă ADN care codează genele IL-12 p35 și p40	Tratamentul cancerului ovarian
Slovak	DNA plazmid kódujúci gény p35 a p40 IL-12	Liečba rakoviny vaječníkov
Slovenian	Plazmid DNA, ki kodira gena IL 12 p35 in p40	Zdravljenje raka na jajčnikih

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Plásmido de ADN que codifica los genes p35 y p40 de IL-12	Tratamiento del cáncer de ovario
Swedish	DNA-plasmid som kodar för IL-12 p35- och p40-gener	Behandling av ovarialcancer
Norwegian	DNA-plasmid som koder for IL-12 p35- og p40-gener	Behandling av eggstokkreft
Icelandic	DNA plasmíð sem kóðar fyrir IL-12 p35 og p40 gen	Meðferð eggjastokkakrabbameins