13 December 2016
EMA/686282/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions, for the treatment of ovarian cancer

On 18 November 2016, orphan designation (EU/3/16/1791) was granted by the European Commission to Dr Ulrich Granzer, Germany, for a vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions (also known as DPX-Survivac) 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 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 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 3.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 170,000 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

*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 513,700,000 (Eurostat 2016).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

patients who have already had initial treatment, the medicine may improve the length of time patients live without their disease getting worse. 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 cells often have increased levels of a protein called survivin, which is involved in the regulation of cell death (apoptosis). In ovarian cancer cells, large amounts of survivin help prevent cell death; this results in the cancer cells surviving much longer than normal cells and multiplying in an uncontrolled fashion.

This medicine works by stimulating the patient's immune system, the body's natural defences, so that it targets and destroys the cancer cells. The medicine contains short fragments of survivin protein. When given to the patient, the patient's immune system is expected to recognise survivin as 'foreign'. This is expected to lead the immune system to attack and destroy ovarian cancer cells overproducing survivin.

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. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 October 2016 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:

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	Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions	Treatment of ovarian cancer
Bulgarian	Ваксина, състояща се от 5 сървайвин пептида с различни рестрикции на човешки левкоцитни антигени	Лечение на рак на яйчниците
Croatian	Cjepivo koje se sastoji od 5 peptida survivina s različitim ograničenjima ljudskih leukocitnih antigena	Liječenje raka jajnika
Czech	Vakcína obsahující 5 survivinových peptidů s různými humánními leukocytárními antigenními restrikcemi	Léčba karcinomu vaječníků
Danish	Vaccine bestående af 5 survivinpeptider med forskellige antigenrestriktioner mod humane leukocytter	Behandling af ovarie cancer
Dutch	Vaccin bestaande uit 5 survivinpeptiden met verschillende humane leukocytenantigeenrestricties	Behandeling van ovariumkanker
Estonian	Viiest surviviini peptiidist, millel on erinevad inimese leukotsüüdi antigeeni piirangud, koosnev vaktsiin	Munasarjavähi ravi
Finnish	Viidestä surviviini-peptidistä, joilla on erilaisia HLA rajoitteita, koostuva rokote	Munasarjasyövän hoito
French	Vaccin consistant en 5 peptides Survivine avec différentes restrictions du système HLA	Traitement du cancer de l'ovaire
German	Vakzin das aus fünf Survivin-Peptiden mit verschiedenen humanen Leukozytenantigen-Restriktionen besteht	Behandlung des Ovarialkarzinoms
Greek	Εμβόλιο που αποτελείται από 5 πεπτίδια σουρβιβίνης με διαφορετικούς περιορισμούς ανθρώπινου λευκοκυτταρικού αντιγόνου	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Öt, eltérő humánleukocitaantigén-restrikciójú szurvivin peptidet tartalmazó oltóanyag	Petefészekrák kezelése
Italian	Vaccino costituito da 5 peptidi di survivina con diversa restrizione per gli antigeni leucocitari umani	Trattamento del carcinoma dell'ovaio
Latvian	Vakcīna, kas sastāv no 5 survivīna peptīdiem ar dažādiem cilvēka leikocītu antigēnu ierobežojumiem	Olnīcu vēža ārstēšana
Lithuanian	Vakcina, sudaryta iš 5 survivino peptidų, turinti skirtingus žmogaus leukocitų antigenų suvaržymus	Kiaušidžių vėžio gydymas
Maltese	Tilqima li tikkonsisti minn 5 peptidi survivin b'restrizzjonijiet differenti ta' antiġen ta' lewkoċiti umani	Kura tal-kanċer ta' l-ovarji

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Szczepionka składająca się z 5 peptydów surwiwiny z różnymi restrykcjami ludzkich antygenów leukocytarnych	Leczenie raka jajnika
Portuguese	Vacina constituída por 5 péptidos da survivina com restrições de antigénios de leucócitos humanos diferentes	Tratamento do carcinoma do ovário
Romanian	Vaccin constând din 5 peptide survivin cu diferite restricții ale antigenului leucocitar uman	Tratamentul cancerului ovarian
Slovak	Vakcína pozostávajúca z 5 survivinových peptidov s rôznymi obmedzeniami ľudských leukocytárnych antigénov	Liečba rakoviny vaječníkov
Slovenian	Cepivo, sestavljeno iz 5 peptidov survivina z različnimi omejitvami humanih levkocitnih antigenov	Zdravljenje raka na jajčnikih
Spanish	Vacuna constituida por 5 péptidos de survivina con diferentes restricciones por antígenos leucocitarios humanos	Tratamiento del cáncer de ovario
Swedish	Vaccin bestående av fem survivinpeptider med olika restriktioner för humant leukocytantigen	Behandling av ovarialcancer
Norwegian	Vaksine som består av 5 survivinpeptider med ulike restriksjoner for humant leukocyttantigen	Behandling av eggstokkreft
Icelandic	Bóluefni sem samanstendur af 5 survívín-peptíðum með mismunandi manna hvítkorna skerðingar	Meðferð eggjastokkakrabbameins