30 March 2015
EMA/COMP/49562/2015
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions for the treatment of ovarian cancer



On 12 February 2015, orphan designation (EU/3/15/1434) was granted by the European Commission to PsiOxus Therapeutics Ltd, United Kingdom, for chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions 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 long-term 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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 early studies in experimental models showed that it

*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 512,900,000 (Eurostat 2015).

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

might have improved effects in terms of slowing down the growth of the cancer, compared with currently authorised 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?

The medicine is made up of an 'oncolytic' virus that it is able to target, infect and destroy cancer cells, but does not infect normal cells. When inside a cancer cell, the virus is expected to take over the cell's replication apparatus and use it to make more copies of itself. This is expected to kill the cell, leaving the virus to spread to neighbouring cancer cells.

## 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.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2015 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:

PsiOxus Therapeutics Ltd
154B Brook Drive
Milton Park
Abingdon
Oxfordshire OX14 4SD
United Kingdom
Tel. +44 123 5835 328
E-mail: enquiries@psioxus.com

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 | Chimeric group B adenovirus (11p/3) with deletions in the E3 and E4 regions | Treatment of ovarian cancer |
| Bulgarian | Химерен аденовирус от група B (11p/3) с делеции в региони E3 и E4 | Лечение на рак на яйчниците |
| Croatian | Kimerični adenovirus skupine B (11p/3) s delecijama u regijama E3 i E4 | Liječenje raka jajnika |
| Czech | Chimerický adenovirus skupiny B (11p/3) s delecemi v oblastech E3 a E4 | Léčba karcinomu vaječníků |
| Danish | Kimærisk gruppe B-adenovirus (11p/3) med deletioner i region E3 og E4 | Behandling af ovarie cancer |
| Dutch | Chimeer groep B adenovirus (11p/3) met deleties in de E3 en E4 regio's | Behandeling van ovariumkanker |
| Estonian | Kimäärne B-rühma adenoviirus (11p/3) deletsioonidega regioonides E3 ja E4 | Munasarjavähi ravi |
| Finnish | Kimeerinen ryhmän B adenovirus (11p/3), jossa deleetioita alueilla E3 ja E4 | Munasarjasyövän hoito |
| French | Adénovirus chimérique du groupe B (11p/3) avec délétions dans les régions E3 et E4 | Traitement du cancer de l'ovaire |
| German | Chimäres Adenovirus der Untergruppe B (11p/3) mit Deletionen in den E3- und E4-Regionen | Behandlung des Ovarialkarzinoms |
| Greek | Χιμαιρικός αδενοϊός ομάδας B (11p/3) με απαλοιφές στις περιοχές E3 και E4 | Θεραπεία του καρκίνου των ωοθηκών |
| Hungarian | Kimérás adenovírus B (11p/3) deléciókkal az E3 és E4 régiókban | Petefészekrák kezelése |
| Italian | Adenovirus chimerico del gruppo B (11p/3) con delezioni nelle regioni E3 ed E4 | Trattamento del carcinoma dell'ovaio |
| Latvian | Himērisks B grupas adenovīruss (11p/3) ar delēcijām E3 un E4 reģionos | Olnīcu vēža ārstēšana |
| Lithuanian | Chimerinis B grupės adenovirusas (11p/3) su E3 ir E4 sričių delecijomis | Kiaušidžių vėžio gydymas |
| Maltese | Adenovirus kimeriku ta' grupp B (11p/3) bi tħassir fir-reġjuni E3 u E4 | Kura tal-kanċer ta' l-ovarji |
| Polish | Adenowirus chimeryczny grupy B (11p/3) z delecjami w regionach E3 i E4 | Leczenie raka jajnika |
| Portuguese | Adenovírus quimérico do grupo B (11p/3) com deleções nas regiões E3 e E4 | Tratamento do carcinoma do ovário |
| Romanian | Adenovirus chimeric de grup B (11p/3) cu deleţii în regiunile E3 şi E4 | Tratamentul cancerului ovarian |
| Slovak | Chimérický adenovírus skupiny B (11p/3) s deléciami v regiónoch E3 a E4 | Liečba rakoviny vaječníkov |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Slovenian | Himerni adenovirus skupine B (11p/3) z delecijami na območjih E3 in E4 | Zdravljenje raka na jajčnikih |
| Spanish | Adenovirus quimérico del grupo B (11p/3) con deleciones en las regiones E3 y E4 | Tratamiento del cáncer de ovario |
| Swedish | Chimärt grupp B-adenovirus (11p/3) med deletioner i E3- och E4-regionerna | Behandling av ovarialcancer |
| Norwegian | Kimært adenovirus gruppe B (11p/3) med delesjoner i E3- og E4-regionen | Behandling av eggstokkreft |
| Icelandic | Blendings-adenóveira í flokki B (11p/3) með úrfellingar á genasvæðum E3 og E4 | Meðferð eggjastokkakrabbameins |