3 May 2017
EMA/143980/2017
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Poly-cyclodextrin-bis-cysteine-PEG$_{3400}$-camptothecin-conjugate for the treatment of ovarian cancer

On 20 March 2017, orphan designation (EU/3/17/1860) was granted by the European Commission to Viadoc Business Solutions Limited, United Kingdom, for poly-cyclodextrin-bis-cysteine-PEG$_{3400}$-camptothecin-conjugate (also known as CRLX101) 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 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 165,000 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 used in combination with other cancer medicines might be of significant benefit in patients whose ovarian cancer did not improve

*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).

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

with authorised 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?

The medicine is made up of a substance called camptothecin, attached to a polymer. Camptothecin blocks an enzyme called topoisomerase I. This enzyme is involved in making copies of DNA (a cell's genetic material) when the cell divides. By blocking the enzyme, the medicine is expected to prevent ovarian cancer cells from dividing and so slow down growth of the cancer.

The polymer is expected to slow down the removal of camptothecin from the body, allowing it to act for longer.

## 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 16 February 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 | Poly-cyclodextrin-bis-cysteine-$PEG_{3400}$-camptothecin-conjugate | Treatment of ovarian cancer |
| Bulgarian | Поли-циклодекстрин-бис-цистеин-$PEG_{3400}$-камптотецин-конюгат | Лечение на рак на яйчниците |
| Croatian | Poli-ciklodekstrin-bis-cistein-$PEG_{3400}$-kamptotekin-konjugat | Liječenje raka jajnika |
| Czech | Poly-cyklodextrin-bis-cystein-$PEG_{3400}$-kamptothecin-konjugát | Léčba karcinomu vaječníků |
| Danish | Poly-cyclodextrin-bis-cystein-$PEG_{3400}$-camptothecin-konjugat | Behandling af ovarie cancer |
| Dutch | Polycyclodextrine-bis-cysteïne-$PEG_{3400}$-camptothecineconjugaat | Behandeling van ovariumkanker |
| Estonian | Polü-tsüklodekstriin-bis-tsüsteiin-$PEG_{3400}$-kamptotetsiin-konjugaat | Munasarjavähi ravi |
| Finnish | Polysyklodekstriinibiskysteiini-$PEG_{3400}$-kamptotesiinikonjugaatti | Munasarjasyövän hoito |
| French | Poly-cyclodextrine-bis-cystéine-$PEG_{3400}$-camptothécine-conjuguée | Traitement du cancer de l'ovaire |
| German | Poly-cyclodextrine-bis-cystein-$PEG_{3400}$-camptothecinkonjugat | Behandlung des Ovarialkarzinoms |
| Greek | Σύζευγμα πολυ-κυκλοδεξτρίνης-δις-κυστεΐνης-$PEG_{3400}$-καμπτοθεκίνης | Θεραπεία του καρκίνου των ωοθηκών |
| Hungarian | Policiklodextrin-bisz-cisztein-$PEG_{3400}$-kamptotecin-konjugátum | Petefészekrák kezelése |
| Italian | Coniugato poli-ciclodestrina-bis-cisteina-$PEG_{3400}$-camptotecina | Trattamento del carcinoma dell'ovaio |
| Latvian | Poli-ciklodekstrīn-bis-cisteīn-$PEG_{3400}$-kamptotecīna konjugāts | Olnīcu vēža ārstēšana |
| Lithuanian | Poli-ciklodekstrino-bis-cisteino-$PEG_{3400}$-kamptotecino konjugatas | Kiaušidžių vėžio gydymas |
| Maltese | Konjugat poly-cyclodextrin-bis-cysteine-PEG3400-camptothecin | Kura tal-kanċer ta' l-ovarji |
| Polish | Koniugat policyklodekstryny-bis-cysteiny-$PEG_{3400}$-kamptotecyny | Leczenie raka jajnika |
| Portuguese | Conjugado de poli-ciclodextrina-bis-cisteína-$PEG_{3400}$-camptotecina | Tratamento do carcinoma do ovário |
| Romanian | Conjugat poli-ciclodextrină-bis-cisteină-$PEG_{3400}$-camptotecină- | Tratamentul cancerului ovarian |
| Slovak | Poly-cyklodextrín-bis-cysteín-$PEG_{3400}$-kamptotecínový konjugát | Liečba rakoviny vaječníkov |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Slovenian | Konjugat poli-ciklodekstrin-bis-cistein-$PEG_{3400}$-kamptotekina | Zdravljenje raka na jajčnikih |
| Spanish | Conjugado de poli-ciclodextrina-bis-cisteína-$PEG_{3400}$-camptotecina | Tratamiento del cáncer de ovario |
| Swedish | Polycyklodextrinbiscystein-$PEG_{3400}$-kamptotesinkonjugat | Behandling av ovarialcancer |
| Norwegian | Poly-syklodekstrin-bis-cystein-$PEG_{3400}$-kamptotecin-konjugat | Behandling av eggstokkreft |
| Icelandic | Pólý-cýklódextrín-bis-cystein-$PEG_{3400}$-kamptótekín konjúgat | Meðferð eggjastokkakrabbameins |