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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate for the treatment of ovarian cancer

On 27 September 2011, orphan designation (EU/3/11/900) was granted by the European Commission to Nektar Therapeutics UK Ltd, United Kingdom, for 20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate for the treatment of ovarian cancer.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries (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 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 is equivalent to a total of around 152,000 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).

[*]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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with ovarian cancer because it is more difficult to break down by the body which prolongs its action in tumour tissues, as supported by early studies, and might improve the outcome of patients. 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?

This medicine is a pro-drug that contains the active substance irinotecan, which has been 'pegylated' (attached to a chemical called polyethylene glycol). When it is given to a patient, the medicine is broken down by the body into irinotecan and a chemical called 7-ethyl-10 hydroxycampothecin (SN38). Irinotecan and SN38 block the action of an enzyme, topoisomerase I, which is involved in the division of cell DNA. When the enzyme is blocked, the DNA strands break, and this prevents the cancer cells from dividing and they eventually die. The fact that the medicine is pegylated is expected to decrease the rate at which the irinotecan is removed from the body, allowing it to act for longer.

What is the stage of development of this medicine?

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

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

At the time of submission, this medicine was not authorised anywhere in the EU for ovarian cancer. Orphan designation of this medicine had been granted in the United States for the treatment of ovarian cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2011 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:

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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	20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate	Treatment of ovarian cancer
Bulgarian	20-пентаеритритол поли (окси-1,2-ethanediyl)-карбоксиметил-glycinate-7-етил-10-хидроксикамптотецин 10-[1,4'-бипиперидин]-1'-карбоксилат	Лечение на рак на яйчниците
Czech	20-pentaerythritol poly (oxy-1,2-ethanediyl)-karboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-karboxylát	Léčba karcinomu vaječníků
Danish	20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinat-7-ethyl-10-hydroxycamptothecin 10-[1,4'-bipiperidin]-1'-carboxylat	Behandling af ovarie cancer
Dutch	20-pentaerytritol poly (oxy-1,2-ethaandiyl)-carboxymethyl-glycinaat-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylaat	Behandeling van ovariumkanker
Estonian	20-pentaerütritooli polü (oksü-1,2-ethanediyl)-karboksümetüül-glycinate-7-etüül-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-karboksülaat	Munasarjavähi ravi
Finnish	20-pentaerytritoli poly (oksi-1,2-etaanidiyyli)-karboksimetyyli-glysinaatti-7-etyyli-10-hydroksikamptotesiini 10-[1,4'-bipiperidiini]-1'-karboksylaatti	Munasarjasyövän hoito
French	20-pentaérythritol poly (oxy-1,2-ethandiyl)-carboxyméthyl-glycinate-7-ethyl-10-hydroxycamptothécine 10-[1,4'-bipipéridine]-1'-carboxylate	Traitement du cancer de l'ovaire
German	20-Pentaerythritol-Poly (oxy-1,2-Ethandiyl)-Carboxymethyl-glycinat-7-Ethyl-10-hydroxycamptothecin 10-[1,4'-bipiperidin]-1'-Carboxylat	Behandlung des Ovarialkarzinoms
Greek	20-πενταερυθρίτης πολυ (οξυ-1,2-αιθανοδιυλ)-καρβοξυμεθυλ-γλυκινικό-7-αιθυλο-10-υδροξυ-καμπτοθεκίνη 10-[1,4'-δι- πιπεριδίνη]-1'-καρβοξυλικό	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	20-pentaeritritol-poli (oxi-1,2-etándiil)-karboximetil-glicinát-7-etil-10-hidroxikaptotecin-10-[1,4'-bipiperidin]-1'-karboxilát	Petefészekrák kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	20-pentaeritritol poli (ossi-1,2-etanediil)-carbossimetil-glicinato-7-etil-10-idrossicamptotecina 10-[1,4'-bipiperidina]-1'-carbossilato	Trattamento del carcinoma dell'ovaio
Latvian	20-pentaeritriola poli (oksi-1,2-etanediil)-karboksimetil-glucināta-7-etil-10-hydroksikamptotecināta 10-[1,4'-bipiperidīna]-1'-karboksilāts	Olnīcu vēža ārstēšanai
Lithuanian	20-pentaeritritolio poli (oksi-1,2-etanediil)-karboksimetil-glicinato-7-etil-10-hidroksikamptotecino 10-[1,4'-bipiperidino]-1'-karboksilatas	Kiaušidžių vėžio gydymas
Maltese	20-pentaerythritol poly(oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate	Kura għal kanċer tal-ovarji
Polish	20-pentaerytrytolu poli(oksy-1,2-etanodiylo)-karboksy-glicynian-7-etylo-10-hydroksykamptotecyno 10-[1,4'-bipiperydyno]-1'-karboksylan	Leczenie raka jajnika
Portuguese	20-pentaeritritol poli (oxi-1,2-etanodiil)-carboximetil-glicinato-7-etil-10-hidroxicamptotecina 10-[bipiperidina-1,4']1'-carboxilato	Tratamento do carcinoma do ovário
Romanian	20-pentaeritritol poli (oxi-1,2-etandiil)-carboximetil-glicinat-7-etil-10-hidroxicamptotecin 10-[bipiperidin 1,4']1'-carboxilat	Tratamentul cancerului ovarian
Slovak	20-pentaerytritol poly (oxy-1,2-etándiyl)-karboxymetyl-glycinát-7-etyl-10-hydroxykamptotecín 10-[1,4'-bipiperidín]-1'-karboxylát	Liečba rakoviny vaječníkov
Slovenian	20-pentaeritritol poli (oksi-1,2-ethanediyl)-karboksimetil-glicinat-7-etil-10-hidroksikamptotekin 10-[1,4'-bipiperidin]-1'-karboksilat	Zdravljenje raka na jajčnikih
Spanish	20-pentaeritritol poli (oxi-1,2-etanodiilo)-carboximetil-glicinato-7-etil-10-hidroxicamptotecina 10-[1-4'-bipiperidina]-1'-carboxilato	Tratamiento del cáncer de ovario
Swedish	20-pentaerytritol-poly (oxi-1,2-etandiyl)-karboximetyl-glycinat-7-etyl-10-hydroxy-kamptotecin 10-[1,4'-bipiperidin]-1'-karboxylat	Behandling av ovarialcancer
Norwegian	20-pentaerytritol poly(oksy-1,2-etandiyl)-karboksymetyl-glysinat-7-etyl-10-hydroksykamptotecin 10-[1,4'-bipiperidin]-1'-karboksylat	Behandling av eggstokkreft
Icelandic	20-pentaerýthrítól fjöl (oxó-1,2-ethanedíýl)-carboxýmethýl-glýcinat-7-etýl-10-hýdroxýcamptóthecín 10-[1,4'-bípiperidín]-1'-carboxýlat	Meðferð eggjastokkakrabbameins