



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂, acetate salt for the treatment of ovarian cancer

On 4 August 2010, orphan designation (EU/3/10/766) was granted by the European Commission to Æterna Zentaris GmbH, Germany, for Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂, acetate salt 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 locally or 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 threshold 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 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂ might be of significant benefit for patients with ovarian cancer because it works in a different way to existing treatments, and early studies indicate

*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 506,500,000 (Eurostat 2010).



that it might improve the treatment of patients with this condition, particularly patients who do not respond to existing 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?

Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂ is made of doxorubicin, a cytotoxic (cell-killing) agent, chemically attached to a modified form of a natural hormone, called luteinising hormone releasing hormone (LHRH, also known as gonadotropin releasing hormone, GnRH). The medicine is expected to work by attaching to the LHRH receptor, which is present on the surface of most ovarian cancer cells. Once attached, the doxorubicin is expected to kill the cancer cells, slowing down the growth and spread 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 Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH₂ 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 the medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 May 2010 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

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Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH ₂ , acetate salt	Treatment of ovarian cancer
Bulgarian	Pyr-His-Trp-Ser-Tyr-D-Lys(доксорубицинилглутарат)-Leu-Arg-Pro-Gly-NH ₂ , ацетатна сол	Лечение на рак на яйчниците
Czech	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinyl-glutarát)-Leu-Arg-Pro-Gly-NH ₂ , acetát	Léčba karcinomu vaječníků
Danish	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarat)-Leu-Arg-Pro-Gly-NH ₂ , acetat salt	Behandling af ovarie cancer
Dutch	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH ₂ , acetaat	Behandeling van ovariumkanker
Estonian	Pyr-His-Trp-Ser-Tyr-D-Lys(doksorubitsinüülgutaraat)-Leu-Arg-Pro-Gly-NH ₂ , atsetaatsool	Munasarjavähi ravi
Finnish	Pyr-His-Trp-Ser-Tyr-D-Lys(doksorubisinyyliglutaraatti)-Leu-Arg-Pro-Gly-NH ₂ , asetaattisuola	Munasarjasyövän hoito
French	Sel d'acétate de Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH ₂	Traitement du cancer de l'ovaire
German	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarat)-Leu-Arg-Pro-Gly-NH ₂ , Azetat	Behandlung des Ovarialkarzinoms
Greek	Pyr-His-Trp-Ser-Tyr-D-Lys(γλουταρική δοξορουμπικίνη)-Leu-Arg-Pro-Gly-NH ₂ , οξικό άλας	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Pir-His-Trp-Ser-Tir-D-Liz (doxorubicinil-glutarát)-Leu-Arg-Pro-Gli-NH ₂ , acetát	Petefészekrák kezelése
Italian	Pir-His-Trp-Ser-Tir-D-Lis(doxorubicinilglutarato)-Leu-Arg-Pro-Gli-NH ₂ , acetato	Trattamento del carcinoma dell'ovaio
Latvian	Pyr-His-Trp-Ser-Tyr-D-Lys(doksorubicinilglutarāts)-Leu-Arg-Pro-Gly-NH ₂ , acetāta sāls	Olnīcu vēža ārstēšana
Lithuanian	Pyr-His-Trp-Ser-Tyr-D-Lys (doksorubicinilglutaratas)-Leu-Arg-Pro-Gly-NH ₂ , acetato druska	Kiaušidžių vėžio gydymas
Maltese	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH ₂ , acetate salt	Kura tal-kanċer ta' l-ovarji
Polish	Octan Pyr-His-Trp-Ser-Tyr-D-Lys(glutarynian doksorubicyny)-Leu-Arg-Pro-Gly-NH ₂	Leczenie raka jajnika
Portuguese	Acetato de Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH ₂	Tratamento do carcinoma do ovário

¹ At the time of designation

Romanian	Acetat de Pir-His-Trp-Ser-Tir-D-Liz (glutarat de doxorubicinil)-Leu-Arg-Pro-Gli-NH ₂	Tratamentul cancerului ovarian
Slovak	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarát)-Leu-Arg-Pro-Gly-NH ₂ , acetát	Liečba rakoviny vaječníkov
Slovenian	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarat)-Leu-Arg-Pro-Gly-NH ₂ , acetatna sol	Zdravljenje raka na jajčnikih
Spanish	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinilglutarato)-Leu-Arg-Pro-Gly-NH ₂ , acetato	Tratamiento del cáncer de ovario
Swedish	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarat)-Leu-Arg-Pro-Gly-NH ₂ , acetatsalt	Behandling av ovarialcancer
Norwegian	Pyr-His-Trp-Ser-Tyr-D-Lys(dokso­rubicinylglutarat)-Leu-Arg-Pro-Gly-NH ₂ , acetatsalt	Behandling av eggstokkreft
Icelandic	Pyr-His-Trp-Ser-Týr-D-Lýs(doxórúbicínýlglútarat)-Leu-Arg-Pró-Glý-NH ₂ , asetat salt	Meðferð eggjastokkakrabbameins

Withdrawal