



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF  
humanised monoclonal antibody to the folate receptor alpha  
for the treatment of ovarian cancer**

On 1 April 2008, orphan designation (EU/3/08/535) was granted by the European Commission to Chiltern International Limited, United Kingdom, for humanised monoclonal antibody to the folate receptor alpha for the treatment of ovarian cancer.

**What is ovarian cancer?**

Tumours that begin in the ovaries are known as ovarian tumours. Tumours which have the potential to grow rapidly and infiltrate surrounding healthy tissues are called ovarian cancers. Due to the absence of symptoms in early stages of the disease, the majority of the patients are diagnosed when the tumours have spread locally or to distant parts of the body. Ovarian cancer is a life-threatening condition.

**What are the methods of treatment available?**

Several anticancer medicinal products were authorised for the condition in the Community at the time of submission of the application for orphan designation. Although a significant proportion of patients respond to the initial chemotherapy (drugs used to kill cancer cells), most ovarian cancers grow again and respond moderately or poorly to subsequent chemotherapy.

Humanised monoclonal antibody to the folate receptor alpha might be of potential significant benefit for the treatment of ovarian cancer. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

**What is the estimated number of patients affected by the condition\*?**

Based on the information provided by the sponsor and previous knowledge of the Committee, ovarian cancer was considered to affect approximately 2.9 in 10,000 persons in the European Union, which, at the time of designation, corresponded to 144,000 persons.

**How is this medicinal product expected to act?**

A large majority of ovarian tumour cells bear a molecule called the folate receptor alpha (a protein that binds folic acid) on the surface of the cell. Humanised monoclonal antibody to the folate receptor alpha attracts normal human blood monocytes, a type of white blood cells that are a part of the immune system. Monocytes are capable of ingesting foreign substances and killing infected cells. The antibody is expected to attract monocytes that may then attack and kill folate receptor alpha-bearing tumour cells, such as cancer cells. This is expected to reduce or to stop the tumour growth.

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\* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**What is the stage of development of this medicinal product?**

The effects of humanised monoclonal antibody to the folate receptor alpha were evaluated in experimental models.

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

Humanised monoclonal antibody to the folate receptor alpha was not authorised anywhere worldwide for treatment of ovarian cancer, at the time of submission. Orphan designation of humanised monoclonal antibody to the folate receptor alpha was granted in the United States for treatment of ovarian cancer.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 6 February 2008 a positive opinion recommending the grant of the above-mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

**For more information:**

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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Humanised monoclonal antibody to the folate receptor-alpha	Treatment of ovarian cancer
Bulgarian	Хуманизирано моноклонално антитяло срещу фолатен рецептор алфа	Лечение на рак на яйчниците
Czech	Humanizovaná monoklonální protilátka proti folátovému alfa receptoru	Léčba karcinomu vaječnicků
Danish	Humaniseret monoklonalt antistof mod folat receptor-alfa	Behandling af ovarie cancer
Dutch	Humaan monoklonaal antilichaam gericht tegen folaatreceptor-alfa	Behandeling van ovariumkanker
Estonian	Humaniseeritud folaadi alfa-retseptori vastane monoklonaalne antikeha	Munasarjavähi ravi
Finnish	Humanisoitu monoklonaalinen vasta-aine folaattireseptori alfalle	Munasarjasyövän hoito
French	Anticorps monoclonal humanisé dirigé contre le récepteur du folate alpha	Traitement du cancer de l'ovaire
German	Humanisierter monoklonaler Antikörper gegen den Folat-Rezeptor alpha	Behandlung des Ovarialkarzinoms
Greek	Ανθρώπειο μονοκλωνικό αντίσωμα εναντίον του υποδοχέα άλφα φολικού οξέος	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	A folát alfa-receptor elleni humanizált monoklonális antitest	Petefészekrák kezelése
Italian	Anticorpo monoclonale umanizzato verso il recettore alfa del folato	Trattamento del carcinoma dell'ovaio
Latvian	Humanizēta monoklonālā anti viela pret folātu alfa-receptoru	Olnīcu vēža ārstēšanai
Lithuanian	Panašūs į žmogaus monokloniniai antikūnai prieš folatų alfa - receptorių	Kiaušidžių vėžio gydymas
Maltese	Anti-korp monoklonali umanizzat għar-riċettur tat-tip alfa tal-folate	Kura tal-kanċer ta' l-ovarji
Polish	Humanizowane monoklonalne przeciwciało przeciw receptorowi alfa kwasu foliowego	Leczenie raka jajnika
Portuguese	Anticorpo monoclonal humanizado contra o receptor alfa dos folatos	Tratamento do cancro do ovário
Romanian	Anticorp monoclonal umanizat anti receptor alfa al folatului	Tratamentul cancerului ovarian
Slovak	Humanizovaná monoklonálna protilátka proti folátovému receptoru-alfa	Liečba rakoviny vaječníkov
Slovenian	Humanizirano monoklonsko protitelo za folatni receptor alfa	Zdravljenje raka na jajčnikih
Spanish	Anticuerpo monoclonal humanizado contra el receptor alfa del folato	Tratamiento del cáncer de ovario
Swedish	Humaniserad monoklonal antikropp till folsyrareceptor-alfa	Behandling av ovarialcancer

Norwegian	Humanisert monoklonalt antistoff mot folatreseptor-alfa	Behandling av eggstokkreft
Icelandic	Mannaaðlagað einstofna mótefni gegn fólatviðtaka-alfa	Meðferð eggjastokkakrabbameins