15 June 2010
EMA/COMP/48649/2008 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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.

The sponsorship was transferred to Eisai Europe Limited, United Kingdom, in June 2010.

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 is the estimated number of patients affected by the condition?

At the time of designation, ovarian cancer affected approximately 2.9 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 144,000 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?

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.

[*]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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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.

How is this medicine expected to work?

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.

What is the stage of development of this medicine?

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.

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

Eisai Europe Limited
European Knowledge Centre
Mosquito Way
Hatfield, Herts AL10 9SN
United Kingdom
Telephone: +44 (0) 845 676 5089
Telefax: +44 (0) 845 676 1401

Patient associations' contact points

Cancer BACUP
3 Bath Place
Rivington Street
London
EC2A 3JR
United Kingdom
Telephone: +44 20 76 96 90 03 / 0808 800 1234 (freephone for UK)
Telefax: +44 20 76 96 90 02

Ligue Nationale contre le Cancer (LNCC)
12, rue Corvisart
75013 Paris
France
Telephone : +33 1 53 55 24 00
Telefax : +33 1 43 36 91 10
E-mail: ligue@ligue-cancer.net

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
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 vaječníků
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 antiviela 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

[1] At the time of designation

Language	Active Ingredient	Indication
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