24 April 2015
EMA/COMP/547302/2008 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Monoclonal antibody against human CD30 for the covalently linked to the cytotoxin monomethylauristatin E for the treatment of Hodgkin lymphoma

First publication	13 May 2009
Rev.1: transfer of sponsorship	7 March 2011
Rev.2: transfer of sponsorship	13 November 2013
Rev.3: sponsor's change of address	24 April 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 15 January 2009, orphan designation (EU/3/08/596) was granted by the European Commission to Seattle Genetics UK Limited, United Kingdom, for monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E for the treatment of Hodgkin lymphoma.

The sponsorship was transferred to Takeda Global Research and Development Centre (Europe) Ltd, United Kingdom, in September 2010 and subsequently to Takeda Pharma A/S, Denmark, in October 2013.

What is Hodgkin lymphoma?

Hodgkin's lymphoma is a type of cancer that develops in the lymphatic system. The lymphatic system is part of the body's immune system: the body's natural defense against infection and disease. It is a complex system made up of organs such as bone marrow (the spongy tissue inside the large bones in the body), thymus and spleen, and a network of lymph nodes throughout the body that are connected by lymphatic vessels. As lymphatic tissue is found throughout the body, Hodgkin's lymphoma can begin in almost any part of the body and can spread to almost any tissue or organ in the body. Normally, the growth and duplication of lymphatic cells takes place in a controlled manner, however in Hodgkin's lymphoma this process is out of control and the cells continue to divide, developing into a tumour. Hodgkin's lymphoma can occur in both adults and children; however, treatment for adults may be different from treatment for children. Hodgkin's lymphoma can usually be cured, if found and

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

An agency of the European Union

© European Medicines Agency, 2015. Reproduction is authorised provided the source is acknowledged.

treated early. Despite the available treatments Hodgkin's lymphoma remains a serious and life threatening condition in certain patients.

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

At the time of designation, Hodgkin lymphoma affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 50,000 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?

Three different types of standard treatment are available and used: chemotherapy (using drugs to kill cancer cells), radiotherapy (using high-energy x-rays or other types of high-energy rays to kill cancer cells) and surgery (removing all possible cancer tissue in an operation). Bone marrow transplantation is also used. Several treatments had been authorized at the time of submission of the application for orphan drug designation.

The monoclonal antibody against CD30 covalently linked to the cytotoxin monomethylauristatin E could be of significant benefit for the treatment of Hodgkin's lymphoma. The main reasons are that it may offer a new way of targeting and killing cancer cells and it might improve the long-term outcome of the patients. 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?

CD30 is a molecule that Hodgkin lymphoma cells have on their surface (surface marker). One part of the medicinal product is an antibody against CD30 (anti-CD30), therefore is able to recognise and bind to CD30. The antibody is linked to a small molecule called monomethyl auristatin E, which is cytotoxic (kills rapidly dividing cancer cells). The CD30 antibody part of the product acts as a carrier for the cytotoxic substance. The product is thought to bind specifically on CD30 receptor of the lymphoma cells. Once bound on the cell surface, it is taken up by the cells. Once inside the cancer cells, the cytotoxic molecule, monomethyl auristatin E, gets released and stops cell division. The cancer cells are then expected to undergo programmed cell death.

What is the stage of development of this medicine?

The effects of monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with Hodgkin's lymphoma had been started.

At the time of submission, the antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E was not authorised anywhere in the world for Hodgkin lymphoma or designated as orphan medicinal product elsewhere for this condition.

[*]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.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 October 2008 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:

Takeda Pharma A/S
Dybendal Alle 10
2630 Taastrup
Denmark
Tel.: +45 46 77 1036
Fax: +45 46 75 6640

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	Monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E	Treatment of Hodgkin lymphoma
Bulgarian	Моноклонално антитяло срещу човешки CD30 антиген, ковалентно свързано с цитотоксина монометилауристатин Е	Лечение на лимфом на Хочкин
Croatian	Monoklonsko protutijelo protiv ljudskog CD30 kovalentno vezanog na citotoksin monometilauristatin E	Liječenje Hodgkinovog limfoma
Czech	Monoklonální anti CD30protilátka kovalentně vázaná na cytotoxin monomethylauristatin E	Léčba Hodgkinova lymfomu
Danish	Monoklonalt antistof mod humant CD30, kovalent bundet til cytotoksinet monomethylauristatin E	Behandling af Hodgkin lymfom
Dutch	Monoklonaal antilichaam gericht tegen humaan CD30, covalent gebonden aan cytotoxine monomethylauristatine E	Behandeling van Hodgkin lymfoom
Estonian	Tsütotoksiin monometüülauristatiin E-ga kovalentselt seotud inimese CD30 vastane monoklonaalne antikeha	Hodgkini lümfoomi ravi
Finnish	Sytotoksiini monometyyliauristatiini E:hen kovalenttisesti kytketty ihmisen monoklonaalinen CD30-vasta-aine	Hodgkinin lymfooman hoito
French	Anticorps monoclonal anti-CD30 humain lié de façon covalente à la cytotoxine monométhylauristatine E	Traitement du lymphome de Hodgkin
German	Gegen humanes CD30-Antigen gerichteter, kovalent an die zytotoxische Substanz Monomethylauristatin E gebundener monoklonaler Antikörper	Behandlung des Hodgkin-Lymphoms
Greek	Μονοκλωνικό αντίσωμα κατά του ανθρώπινου CD30 ομοιοπολικά συνδετού με την κυτταροτοξίνη μονομεθυλαυριστατίνη Ε	Θεραπεία του λεμφώματος Hodgkin
Hungarian	A monometil-aurisztatin E citotoxinhoz kovalensen kötött, humán CD30 elleni monoklonális antitest	Hodgkin lymphoma kezelése
Italian	Anticorpo monoclonale anti-CD30 umano legato in modo covalente alla citotossina monometilauristatina E	Trattamento del linfoma di Hodgkin

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Latvian	Monoklonālās antivielas pret cilvēka CD30, kovalenti saistītas ar citotoksīnu monometilauristatīnu E	Hodžkina limfomas ārstēšana
Lithuanian	Monokloninis antikūnas prieš žmogaus CD30, kovalentiniu ryšiu sujungtas su monometilauristatino E citotoksinu	Hodžkino limfomos gydymas
Maltese	Antikorp monoklonali kontra CD30 uman marbut b'mod kovalenti maċ-ċitotossina monomethylauristatin E	Kura tal-limfoma ta' Hodgkin
Polish	Przeciwciało monoklonalne przeciwko ludzkiemu CD30, związane kowalencyjnie z cytotoksyczną monometyloauristatyną E	Leczenie chłoniaka Hodgkina (ziarnicy złośliwej)
Portuguese	Anticorpo monoclonal anti-CD30 humano ligado de forma covalente à citotoxina monometilauristatina E	Tratamento do linfoma de Hodgkin
Romanian	Anticorp monoclonal împotriva CD30 uman, legat covalent la citotoxina monometil-auristatină E	Tratamentul limfomului Hodgkin
Slovak	Monoklonálna protilátka proti ľudskému CD30, kovalentne naviazaná na cytotoxín monometylauristatín E	Liečba lymfómu Hodgkinovho typu
Slovenian	Monoklonsko protitelo proti humanemu CD30, kovalentno vezano na citotoksin monometilavristatin E	Zdravljenje Hodgkinovega limfoma
Spanish	Anticuerpo monoclonal anti-CD30 humano, unido covalentemente a la citotoxina monometilauristatina E	Tratamiento del linfoma de Hodgkin
Swedish	Monoklonal antikropp mot humant CD30 kovalent bunden till cytotoxinet monometylauristatin E	Behandling av Hodgkin lymfom
Norwegian	Monoklonalt antistoff mot humant CD30 kovalent bundet til cytotoksinet monometylauristatin E	Behandling av Hodgkin-lymfom
Icelandic	Einstofna mótefni gegn manna-CD30 tengda frumueitrinu mónómetýlauristatíni E með jafngildum (covalent) tengjum	Meðferð við Hodgkins sjúkdómi