



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 29 July 2008
Doc.Ref.: EMEA/COMP/56003/2006 Rev.1

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2008 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF human monoclonal antibody against HLA-DR for the treatment of multiple myeloma

On 28 February 2006, orphan designation (EU/3/06/358) was granted by the European Commission to GPC Biotech AG, Germany, for human monoclonal antibody against HLA-DR for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cells called plasma cells. Plasma cells are found in the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells called “blasts” that mature into several different types of blood cells that have specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. Platelets make the blood clot, and white blood cells fight infection. In multiple myeloma an excessive number of plasma cells are produced. Normally, the division of cells takes place in a controlled manner but with multiple myeloma, the process gets out of control and abnormal plasma cells multiply, producing many myeloma cells. These fill up the bone marrow and interfere with production of the normal white cells, red cells and platelets. This leads to a number of possible complications, which include anaemia, bone pain and fractures, raised levels of calcium in the blood and kidney disease. Multiple myeloma is life-threatening.

What are the methods of treatment available?

The main treatment of multiple myeloma is chemotherapy (using drugs to kill cancer cells) usually combined with steroids (a group of chemical substances, belonging to the so-called hormones, which have an effect on the activity of certain organs). Other types of treatment for multiple myeloma are radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) and immunotherapy (using drugs that stimulate the body’s own immune system to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

Human monoclonal antibody against HLA-DR could be of potential significant benefit for the treatment of multiple myeloma because it may act in a different way than other available medicines and thereby 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.

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, multiple myeloma was considered to affect less than 1.9 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 87,000 persons.

How is this medicinal product expected to act?

Antibodies are proteins in the body that target and bind specific structures on the surface of foreign bodies, such as bacteria or cancer cells. Human monoclonal antibody against HLA-DR is an antibody that recognises a specific area of human leucocyte antigen-DR (HLA-DR), which is a protein present in the surface of some cells in the body, belonging to the immune system. In experimental conditions it has been shown that when the antibody binds the HLA-DR on malignant cells, it triggers their death. Through this mechanism human monoclonal antibody against HLA-DR might induce the death of multiple myeloma cells.

What is the stage of development of this medicinal product?

The effects of human monoclonal antibody against HLA-DR were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials including patients with multiple myeloma were started.

Human monoclonal antibody against HLA-DR was not authorised anywhere worldwide for multiple myeloma, at the time of submission. Orphan designation of human monoclonal antibody against HLA-DR was granted in the EU for the treatment of Hodgkin's lymphoma.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 February 2006 a positive opinion recommending the grant of the above-mentioned designation.

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:

Sponsor's contact details:

GPC Biotech AG

Fraunhoferstrasse 20

D-82152 Martinsried

Munich

Germany

Telephone: +49 89 85 65 32 10

Telefax: +49 89 85 65 26 97

E-mail: info@gpc-biotech.com

* 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 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Patients' associations contact points:

International Myeloma Foundation (UK)

Lower Ground Floor

37 York Place

Edinburgh

Scotland

EH1 3HP

United Kingdom

Myeloma Infoline: 0800 980 3332 (freephone for UK)

Telephone: +44 13 15 57 33 32 (Administration)

Telefax: +44 13 15 56 97 20

E-mail: TheIMF@myeloma.org.uk

Danish Myeloma Association

(Dansk Myelomatose Forening)

v/ Peter Randløv

Klosterbakken 40

DK-3500 Værløse

Denmark

Telephone: +45 44 48 41 27

E-mail: randlov@post9.tele.dk

Arbeitsgemeinschaft Multiples Myelom/Plasmozytom (APMM)

Freinbergstraße 12

4020 Linz

Austria

Telephone : +43 73 27 73 465

Telefax: +43 73 27 73 464

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

Language	Active Ingredient	Indication
English	Human monoclonal antibody against HLA-DR	Treatment of multiple myeloma
Czech	Lidská monoklonální protilátka proti HLA-DR	Léčba mnohočetného myelomu
Danish	Humant monoklonalt antistof mod HLA-DR	Behandling af multipelt myelom
Dutch	Humaan monoklonaal antilichaam tegen HLA-DR	Behandeling van multipel myeloom
Estonian	HLA-DR vastane inimese monoklonaalne antikeha	Multiibelse imüeloomi ravi
Finnish	Humanisoitu monoklonaalinen HLA-DR-vasta-aine	Multippeli myelooman hoito
French	Anticorps monoclonal humain contre HLA-DR	Traitement du myélome multiple
German	Humaner monoklonaler Antikörper gegen HLA-DR	Behandlung des multiplen Myeloms
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα κατά HLA-DR	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	HLA-DR elleni humán monoklonális ellenanyag	Myeloma multiplex kezelése
Italian	Anticorpo monoclonale umano contro l'HLA-DR	Trattamento del mieloma multiplo
Latvian	Cilvēka monoklonālās antivielas pret HLA-DR	Multiplās mielomas ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas prieš ŽLA-DR	Dauginės mielomos gydymas
Polish	Ludzkie przeciwciało monoklonalne przeciwko HLA-DR	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal humano contra o HLA-DR	Tratamento do mieloma múltiplo
Slovak	Ľudské monoklonné protilátky proti HLA-DR	Liečba mnohopočetného myelómu
Slovenian	Človeška monoklonska protitelesa proti HLA-DR	Zdravljenje multiplega mieloma
Spanish	Anticuerpo monoclonal humano contra HLA-DR	Tratamiento del mieloma múltiple
Swedish	Human monoklonal antikropp mot HLA-DR	Behandling av multipelt myelom
Norwegian	Humant monoklonalt antistoff mot HLA-DR	Behandling av myelomatose
Icelandic	Einstofna mótefni úr mönnum gegn HLA-DR mótefnavökum	Meðferð við mergfrumuæxli