



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

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EMA/COMP/11030/03

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in November 2004 on request from the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF humanised anti-HM1.24 monoclonal antibody for the treatment of multiple myeloma

On 10 May 2001, orphan designation EU/3/01/037 was granted by the European Commission to Chugai Pharma Europe Ltd., United Kingdom, for humanised anti-HM1.24 monoclonal antibody for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called a plasma cell. 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 the production of 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, the so-called hormones, which have an effect on the activity of certain organs). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Radiotherapy can be very useful to treat painful areas and weakened bones. Interferon alpha is a protein normally produced by the body in response to viral infections, such as flu. Sometimes a combination of interferon and chemotherapy may be used.

Humanised anti-HM1.24 monoclonal antibody could be of potential significant benefit for the treatment of multiple myeloma. The main reasons are that it may offer a new way of 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.

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

According to the information provided by the sponsor, multiple myeloma was considered to affect about 62,000 in the European Union.

How is this medicinal product expected to act?

Antibodies are proteins in the body that target specific shapes (the so-called antigens) on the surface of foreign bodies, such as bacteria or virus. The antibody will lock on to, and react with, the foreign antigen, leading to the destruction of the bacteria or virus. Humanised anti-HM1.24 monoclonal antibody is supposed to recognise the HM1.24 antigen on the myeloma cells and lock on to it (like a key in a lock). This link would then trigger the body's immune system to attack the myeloma cancer cells or even induce the cancer cells to destroy themselves.

What is the stage of development of this medicinal product?

The effects of humanised anti-HM1.24 monoclonal antibody were evaluated in experimental models. At the time of submission of the application for orphan designation, one clinical trial in patients with multiple myeloma was on going.

Humanised anti-HM1.24 monoclonal antibody was not marketed or designated as orphan medicinal product elsewhere, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 20 March 2001 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:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Humanised Anti-HM1.24 Monoclonal Antibody	Treatment of multiple myeloma
Danish	Humaniseret Anti-HM1.24 Monoklonalt Antistof	Behandling af multipelt myelom
Dutch	Gehumaniseerd Anti-HM1.24 Monoclonaal Antilichaam	Behandeling van multipel myeloom
Finnish	Humaani anti-HM1.24 monoklonaalinen vasta-aine	Multippelin myelooman hoito
French	Anticorps Monoclonal Anti- HM1.24 Humanisé	Traitement du myélome multiple
German	Humanisierter Anti-HM1.24- monoklonaler Antikörper	Behandlung des multiplen Myeloms
Greek	Ανθρώποποιημένο μονοκλωνικό Αντίσωμα Αντι-HM1.24	Θεραπεία του πολλαπλού μυελώματος
Italian	Anticorpo monoclonale umanizzato anti-HM1.24	Trattamento del mieloma multiplo
Portuguese	Anticorpo monoclonal anti- HM1.24 humanizado	Tratamento do mieloma múltiplo
Spanish	Anticuerpo monoclonal anti- HM1.24 humanizado	Tratamiento del mieloma múltiple
Swedish	Humaniserad anti-HM1.24 monoklonal antikropp	Behandling av multipelt myelom