



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of

milatuzumab for the treatment of multiple myeloma

On 19 January 2009, orphan designation (EU/3/08/601) was granted by the European Commission to Immunomedics GmbH, Germany, for milatuzumab for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells are found in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes out of control, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of the normal white cells, red cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease. Multiple myeloma is a life-threatening disease.

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

At the time of designation multiple myeloma affected approximately 2 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 100,000 people.

What treatments are available?

At the time of submission of the application for orphan drug designation, several medicines were authorised for multiple myeloma in the European Union. The main treatment for multiple myeloma is chemotherapy (medicines to treat cancer) usually combined with steroids (a group of chemical substances, the so-called hormones, which have an effect on the activity of certain organs). Interferon alfa can also be used in combination with chemotherapy although the way it works in cancer treatment is not fully understood. Radiotherapy (using radiation to kill cancer cells) can be very useful to treat pain and weakened bones.

The sponsor has provided sufficient information to show that milatuzumab might be of significant benefit for patients because it works in a different way to other medicines and might be able to improve the long-term outcome of patients with multiple myeloma. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

* 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 Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

How is this medicine expected to work?

Milatumuzumab is a monoclonal antibody (a type of protein) that has been designed to recognise and bind to a specific structure (called an antigen) called CD74. This is a receptor protein that is often found on the surface of multiple myeloma cells and is involved in the growth and survival of the cells. By attaching itself to the CD74 on cancer cells, milatumuzumab is expected to stop their development and cause the cells to die.

What is the stage of development of this medicine?

The effects of milatumuzumab have been evaluated in experimental models.

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

At the time of submission, milatumuzumab was not authorised anywhere in the world for multiple myeloma. Orphan designation of milatumuzumab had been granted in the United States of America for multiple myeloma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 November 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;
- and either the rarity of the condition (affecting not more than 5 in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:

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Withdrawal

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

| Language | Active Ingredient | Indication |
|-----------------|--------------------------|----------------------------------------|
| English | Milatumzumab | Treatment of multiple myeloma |
| Bulgarian | милатуцумаб | Лечение на мултиплен миелом |
| Czech | Milatuzumab | Léčba mnohočetného myelomu |
| Danish | Milatuzumab | Behandling af multipelt myelom |
| Dutch | Milatuzumab | Behandeling van multipel myeloom |
| Estonian | Milatuzumab | Multiibelse imüeloomi ravi |
| Finnish | Milatuzumabia | Multippeli myelooman hoito |
| French | Milatuzumab | Traitement du myélome multiple |
| German | Milatuzumab | Behandlung des multiplen Myeloms |
| Greek | μιλατουζουμάμπη | Θεραπευτική αγωγή πολλαπλού μυελώματος |
| Hungarian | Milatuzumab | Myeloma multiplex kezelése |
| Italian | Milatuzumab | Trattamento del mieloma multiplo |
| Latvian | Milatuzumabs | Multiplās mielomas ārstēšana |
| Lithuanian | Milatuzumabas | Dauginės mielomos gydymas |
| Maltese | Milatuzumab | Kura tal-mjeloma multipla |
| Polish | Milatuzumabu | Leczenie szpiczaka mnogiego |
| Portuguese | Milatuzumab | Tratamento do mieloma múltiplo |
| Romanian | Milatuzumab | Tratamentul mielomului multiplu |
| Slovak | Milatuzumab | Liečba mnohopočetného myelómu |
| Slovenian | Milatuzumab | Zdravljenje multiplega mieloma |
| Spanish | Milatuzumab | Tratamiento del mieloma múltiple |
| Swedish | Milatuzumab | Behandling av multipelt myelom |
| Norwegian | Milatuzumab | Behandling av myelomatose |
| Icelandic | Mílatuzúmab | Meðferð við mergfrumuæxli |