



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

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## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of human anti-intercellular adhesion molecule-1 monoclonal antibody for the treatment of multiple myeloma**

On 20 January 2009, orphan designation (EU/3/08/600) was granted by the European Commission to BioInvent International AB, Sweden, for human anti-intercellular adhesion molecule-1 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 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 is the estimated number of patients affected by the condition?**

At the time of designation multiple myeloma affected approximately 1.3 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 50,000 people.

#### **What treatments are available?**

Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. The main treatment for multiple myeloma is chemotherapy (medicines to treat cancer), which is usually combined with steroids (a group of chemical substances, the so-called hormones, which have an effect on the activity of certain organs). Other types of treatment for multiple myeloma are radiotherapy (using radiation to kill cancer cells) and immunotherapy (using drugs that stimulate the body's own immune system to kill cancer cells). Radiotherapy can be useful to treat painful areas and weakened bones. Human anti-intercellular adhesion molecule-1 monoclonal antibody could be of potential significant benefit for the treatment of multiple myeloma because it may act in a different way to other available

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\* 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).

medicines. This assumption will have to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

#### **How is this medicine expected to work?**

Human anti-intercellular adhesion molecule-1 monoclonal antibody is a monoclonal antibody (a type of protein) that has been designed by the sponsor to recognise and bind to a specific structure (called an antigen) called 'intercellular adhesion molecule-1' (also known as CD54). This is a protein that is found on the surface of the cancer cells in multiple myeloma and is involved in the growth, survival and spread of multiple myeloma. By attaching itself to this protein, human anti-intercellular adhesion molecule-1 monoclonal antibody might stop the development of the disease.

#### **What is the stage of development of this medicine?**

The effects of human anti-intercellular adhesion molecule-1 monoclonal antibody have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with multiple myeloma had been initiated.

Human anti-intercellular adhesion molecule-1 monoclonal antibody was not authorised anywhere worldwide for treatment of multiple myeloma or designated as orphan medicinal product elsewhere for this condition at the time of submission.

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.

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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 five in 10,000 people in the Community) 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.

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

| <b>Language</b> | <b>Active Ingredient</b>                                                              | <b>Indication</b>                      |
|-----------------|---------------------------------------------------------------------------------------|----------------------------------------|
| English         | Human anti-intercellular adhesion molecule-1 monoclonal antibody                      | Treatment of multiple myeloma          |
| Bulgarian       | Човешко анти-вътреклетъчна адхезионна молекула-1 моноклонално антитяло                | Лечение на мултиплен миелом            |
| Czech           | Lidská monoklonální protilátka anti intercelulární adhezivní molekula-1               | Léčba mnohočetného myelomu             |
| Danish          | Humant anti-intercellulært adhæsiionsmolekyle-1 monoklonalt antistof                  | Behandling af multipelt myelom         |
| Dutch           | Humaan anti-intercellulaire adhesiemolecule-1 monoclonaal antilichaam                 | Behandeling van multipel myeloom       |
| Estonian        | Inimese intertsellulaarse adhesioonimolekuli-1 vastane monoklonaalne antikeha         | Multiibelse müeloomi ravi              |
| Finnish         | Ihmisen anti-intersellulaarisen adheesiomolekyyli-1:n monoklonaalinen vasta-aine      | Multippeli myelooman hoito             |
| French          | Anticorps monoclonal humain dirigé contre la molécule d'adhésion intercellulaire-1    | Traitement du myélome multiple         |
| German          | Humaner anti-ICAM-1-monoklonaler Antikörper                                           | Behandlung des multiplen Myeloms       |
| Greek           | Ανθρώπινο μονοκλωνικό αντίσωμα έναντι μεσοκυττάριου μορίου προσκόλλησης-1             | Θεραπευτική αγωγή πολλαπλού μυελώματος |
| Hungarian       | Humán intracelluláris adhéziós molekula-1 ellenes monoclonalis antitest               | Myeloma multiplex kezelése             |
| Italian         | Anticorpo monoclonale umano diretto contro la molecola-1 di adesione intercellulare   | Trattamento del mieloma multiplo       |
| Latvian         | Cilvēka monoklonālās antivielas pret starpšūnu adhēzijas molekulu-1                   | Multiplās mielomas ārstēšana           |
| Lithuanian      | Žmogaus monokloninis antikūnas prieš tarpląstelinę adhezijos molekulę - 1             | Dauginės mielomos gydymas              |
| Maltese         | Antikorp monoklonali uman kontra l-molekola-1 ta' adeżjoni interċellulari             | Kura tal-mjeloma multipla              |
| Polish          | Ludzkie monoklonalne przeciwciało przeciwko wewnątrzkomórkowej molekuły adhezyjnej -1 | Leczenie szpiczaka mnogiego            |
| Portuguese      | Anticorpo monoclonal humano anti-molécula de adesão intercelular-1                    | Tratamento do mieloma múltiplo         |
| Romanian        | Anticorp monoclonal uman anti molecula de adeziune intercelulară-1                    | Tratamentul mielomului multiplu        |
| Slovak          | Ľudská monoklonálna protilátka proti anti-intercelulárnej adhezívnej molekule-1       | Liečba mnohopočetného myelómu          |
| Slovenian       | Humano monoklonalno protitelo za anti-intercelularno adhezijsko molekulo-1            | Zdravljenje multiplega mieloma         |
| Spanish         | Anticuerpo monoclonal humano contra la molécula de adhesión intercelular-1            | Tratamiento del mieloma múltiple       |
| Swedish         | Mänsklig anti-intercellulär adhesionsmolekyl-1 monoklonal antikropp                   | Behandling av multipelt myelom         |

|           |                                                                    |                           |
|-----------|--------------------------------------------------------------------|---------------------------|
| Norwegian | Humant anti-intercellular adhesjonsmolekyl-1 monoklonalt antistoff | Behandling av myelomatose |
| Icelandic | Manna einstofna mótefni gegn innanfrumu viðloðunarbindli-1         | Meðferð við mergfrumuæxli |