



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

17-allylamino-17-demethoxygeldanamycin for the treatment of multiple myeloma

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2011 on request of the Sponsor.***

On 21 December 2004, orphan designation (EU/3/04/247) was granted by the European Commission to Wainwright Associates Limited, United Kingdom, for 17-allylamino-17-demethoxygeldanamycin for the treatment of multiple myeloma.

The sponsorship was transferred to Bristol-Myers Squibb International Corporation, Belgium, in October 2008.

### 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 in 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 1.2 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 55,000 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?**

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). Other types of treatments 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). Radiotherapy can be useful to treat painful areas and weakened bones. Several products were authorised for the condition in the Community at the time of submission of the application for orphan designation.

17-allylamino-17-demethoxygeldanamycin could be of potential significant benefit for the treatment of multiple myeloma because it may act in a different way than other available medicines. 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?**

17-allylamino-17-demethoxygeldanamycin is an antibiotic, which is expected to act by inhibiting a natural cell molecule called heat shock protein 90. Heat-shock proteins are cellular natural proteins which are important for regulating some basic cellular activities, such as proteins production and trafficking. In particular, heat-shock proteins are triggered by various factors menacing cell survival. It is assumed that by blocking the heat-shock protein 90 17-allylamino-17-demethoxygeldanamycin will stop cancer cell division and trigger cancer cell death in multiple myeloma.

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

The effects of 17-allylamino-17-demethoxygeldanamycin were evaluated in experimental models.

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

17-allylamino-17-demethoxygeldanamycin was not marketed anywhere worldwide for treatment of multiple myeloma. Orphan designation 17-allylamino-17-demethoxygeldanamycin was granted in the United States for the treatment of multiple myeloma, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 November 2004 recommending the granting of this designation.

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\*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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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:

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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<sup>1</sup>, Norwegian and Icelandic

| Language   | Active Ingredient                          | Indication                             |
|------------|--------------------------------------------|----------------------------------------|
| English    | 17-allylamino-17-demethoxygeldanamycin     | Treatment of multiple myeloma          |
| Bulgarian  | 17-алиламино-17-деметоксигелданамицин      | Лечение на мултиплен миелом            |
| Czech      | 17-alylamino-17-demethoxygeldanamycin      | Léčba mnohočetného myelomu             |
| Danish     | 17-allylamino-17-demethoxygeldanamycin     | Behandling af multipelt myelom         |
| Dutch      | 17-allylamino-17-demethoxygeldanamycine    | Behandeling van multipel myeloom       |
| Estonian   | 17-allüüламиин-17-demetoksügeldanamütsiin  | Multiibelse imüeloomi ravi             |
| Finnish    | 17-allyyliamino-17-dimetoksigeldanamysiini | Multippeli myelooman hoito             |
| French     | 17-allylamino-17-déméthoxygeldanamycine    | Traitement du myélome multiple         |
| German     | 17-Allylamin-17-demethoxygeldanamycin      | Behandlung des multiplen Myeloms       |
| Greek      | 17-αλλυλαμινό-17-δεμεθοξυγελδαναμυκίνη     | Θεραπευτική αγωγή πολλαπλού μυελώματος |
| Hungarian  | 17-allilamino-17-dimetoxi-geldanamycin     | Myeloma multiplex kezelése             |
| Italian    | 17-amminoallil-17-demetossigeldanamicina   | Trattamento del mieloma multiplo       |
| Latvian    | 17-alilamino-17-demetoksigeldamicīns       | Multiplās mielomas ārstēšana           |
| Lithuanian | 17-alilamino-17-demetoksigeldanamicinas    | Dauginės mielomos gydymas              |
| Maltese    | 17-allylamino-17-demethoxygeldanamycin     | Kura tal-mjeloma multipla              |
| Polish     | 17-alliloamino-17-demetoksygeldanamycyna   | Leczenie szpiczaka mnogiego            |
| Portuguese | 17-alilamino-17-demetoxigeldanamicina      | Tratamento do mieloma múltiplo         |
| Romanian   | 17-alilamino-17-demetoxi geldanamicină     | Tratamentul mielomului multiplu        |
| Slovak     | 17-alylamino-17-demetoxygeldanamycín       | Liečba mnohopočetného myelómu          |
| Slovenian  | 17-alilamino-17-demetoksigeldanamicin      | Zdravljenje multiplega mieloma         |
| Spanish    | 17-alilamino-17-demetoxigeldanamicina      | Tratamiento del mieloma múltiple       |
| Swedish    | 17-allylamin-17-demetoxigeldanamycin       | Behandling av multipelt myelom         |
| Norwegian  | 17-allylamino-17-demetoksygeldanamycin     | Behandling av myelomatose              |
| Icelandic  | 17-allýlamínó-17-demetoxýgeldanamýsín      | Meðferð við mergfrumuæxli              |

<sup>1</sup> At the time of transfer of sponsorship