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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

17-allylamino-17-demethoxygeldanamycin for the treatment of chronic myeloid leukaemia

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2011 on request of the Sponsor.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 26 January 2005, orphan designation (EU/3/04/256) was granted by the European Commission to Wainwright Associates Limited, United Kingdom, for 17-allylamino-17-demethoxygeldanamycin for the treatment of chronic myeloid leukaemia.

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

What is chronic myeloid leukaemia?

Chronic myeloid leukaemia is a disease in which cancer cells are found in the blood and 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. White blood cells fight infection. Platelets make the blood clot. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukaemias. In myeloid leukaemia blasts that are developing into white blood cells called granulocytes, are affected. The blasts do not mature and become too many. These blast cells are then found in the blood and also accumulate in the bone marrow where they take the place of the other normal types of blood cells. The disease can develop very slowly, which is why it is called "chronic" myeloid leukaemia. Chronic myeloid leukaemia is life-threatening.

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

At the time of designation, chronic myeloid leukaemia affected approximately 0.5 in 10,000 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 23,000 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?

Treatment for leukaemia is complex and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. At the time of submission of the application for orphan drug designation authorised treatments of chronic myeloid leukaemia included chemotherapy agents (using drugs to kill cancer cells) and immunotherapy agents (using drugs that stimulate the body's own immune system to kill cancer cells). Sometimes a combination of immunotherapy and chemotherapies may have been used. Furthermore, there exists another product with another mechanism of action that works by blocking (inhibiting) growth signals within cancer cells and preventing a series of chemical reactions that stimulates cell growth and division, the final result being that cancer cells stop growing. Bone marrow transplantation was also used.

17-allylamino-17-demethoxygeldanamycin could be of potential significant benefit for the treatment of chronic myeloid leukaemia 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 acts by inhibiting a molecule called a heat-shock protein 90. Heat-shock proteins are a group of molecules triggered by various factors such as stress and cancer genes. It is assumed that by blocking the heat-shock protein 90, 17-allylamino-17-demethoxygeldanamycin will block cancer cell division and trigger cancer cell death in chronic myeloid leukaemia.

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 chronic myeloid leukaemia were ongoing.

17-allylamino-17-demethoxygeldanamycin was not marketed anywhere worldwide for treatment of chronic myeloid leukaemia at the time of submission. Orphan designation of 17-allylamino-17-demethoxygeldanamycin was granted in the United States for the treatment of chronic myeloid leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2004 recommending the granting of this designation.

*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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	17-allylamino-17-demethoxygeldanamycin	Treatment of chronic myeloid leukaemia
Bulgarian	17-алиламино-17-деметоксигелданамицин	Лечение на хронична миелоидна левкемия
Czech	17-alylamino-17-demethoxygeldanamycin	Léčba chronické myeloidní leukémie
Danish	17-allylamino-17-demethoxygeldanamycin	Behandling af kronisk myeloid leukæmi
Dutch	17-allylamino-17-demethoxygeldanamycine	Behandeling van chronische myeloïde leukemie
Estonian	17-allüламиин-17-demetoksügeldanamütsiin	Kroonilise müeloidse leukeemia ravi
Finnish	17-allyyliamino-17-dimetoksigeldanamysiini	Kroonisen myelooisen leukaemian hoito
French	17-allylamino-17-déméthoxygeldanamycine	Traitement de la leucémie myéloïde chronique
German	17-Allylamin-17-demethoxygeldanamycin	Behandlung der chronischen myeloischen Leukämie
Greek	17-αλλυλαμινό-17-δεμεθοξυγελδαναμυκίνη	Θεραπεία της χρόνιας μυελοειδούς λευχαιμίας
Hungarian	17-allilamino-17-dimetoxi-geldanamycin	Krónikus myeloid leukémia kezelése
Italian	17-amminoallil-17-demetossigeldanamicina	Trattamento della leucemia mieloide cronica
Latvian	17-alilamino-17-demetoksigeldamicīns	Hroniskas mieloleikozes ārstēšana
Lithuanian	17-alilamino-17-demetoksigeldanamicinas	Lėtinės mielocitinės leukemijos gydymas
Maltese	17-allylamino-17-demethoxygeldanamycin	Kura tal-lewkimja mjelojda kronika
Polish	17-alliloamino-17-demetoksygeldanamycyna	Leczenie przewlekłej białaczki szpikowej
Portuguese	17-alilamino-17-demetoxigeldanamicina	Tratamento da leucemia mielóide crónica
Romanian	17-alilamino-17-demetoxi geldanamicină	Tratamentul leucemiei mieloide cronice
Slovak	17-alylamino-17-demetoxygeldanamycín	Liečba chronickej myeloidnej leukémie
Slovenian	17-alilamino-17-demetoksigeldanamicin	Zdravljenje kronične mieloične levkemije
Spanish	17-alilamino-17-demetoxigeldanamicina	Tratamiento de la leucemia mieloide crónica

¹ At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Swedish	17-allylamin-17-demetoxigeldanamycin	Behandling av kronisk myeloid leukemi
Norwegian	17-allylamino-17-demetoksygeldanamycin	Behandling av kronisk myelogen leukemi
Icelandic	17-allýlamínó-17-demetoxýgeldanamýsín	Meðferð við langvinnu hvítblæði í beinmerg