



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Bosutinib for the treatment of chronic myeloid leukaemia

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Rev.1: transfer of sponsorship	16 June 2011
Rev.2: information about Marketing Authorisation	3 May 2013
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 4 August 2010, orphan designation (EU/3/10/762) was granted by the European Commission to Wyeth Europa Limited, United Kingdom, for bosutinib for the treatment of chronic myeloid leukaemia.

The sponsorship was transferred to Pfizer Limited, United Kingdom, in May 2011.

What is chronic myeloid leukaemia?

Chronic myeloid leukaemia (CML) is a cancer of the white blood cells (cells that fight against infections). In patients with CML, the bone marrow (the spongy tissue inside the large bones) produces large numbers of abnormal, immature white blood cells called 'blasts', so there are too many of them circulating in the blood. These blast cells are not fully developed and do not work properly. Over a period of time, they replace the normal white blood cells, red blood cells and platelets in the bone marrow.

CML is most common in adults and older people, but children may also be affected. The disease usually develops very slowly, which is why it is called 'chronic'. However, when it progresses, CML is a severe and life-threatening disease that is associated with poor overall survival.

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

At the time of designation, CML affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 81,000 people*, and is below the threshold for orphan

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).



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 CML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatments for CML were chemotherapy (medicines to treat cancer) and bone marrow transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from a matched donor).

The sponsor has provided sufficient information to show that bosutinib might be of significant benefit for patients with CML because early studies indicate that it might improve the treatment of patients who do not respond to existing treatments. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Bosutinib is expected to work by blocking two types of enzymes called Src and Abl tyrosine kinases. These enzymes can be found in some receptors on the surface of leukaemia cells, including the receptors that are involved in stimulating the cells to divide uncontrollably. By blocking these enzymes, bosutinib is expected to control the spread of leukaemia cells.

What is the stage of development of this medicine?

The effects of bosutinib have been evaluated in experimental models.

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

At the time of submission, bosutinib was not authorised anywhere in the EU for CML. Orphan designation of bosutinib had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 May 2010 recommending the granting of this designation.

Update: Bosutinib (Bosulif) has been authorised in the EU since 27 March 2013 for the treatment of adult patients with chronic-phase, accelerated-phase and blast-phase Philadelphia-chromosome-positive chronic myelogenous leukaemia previously treated with one or more tyrosine kinase inhibitors and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options.

More information on Bosulif can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find_medicine/Human_medicines/European_Public_Assessment_Reports

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	Bosutinib	Treatment of chronic myeloid leukaemia
Bulgarian	Босутиниб	Лечение на хронична миелоидна левкемия
Czech	Bosutinib	Léčba chronické myeloidní leukémie
Danish	Bosutinib	Behandling af kronisk myeloid leukæmi
Dutch	Bosutinib	Behandeling van chronische myeloïde leukemie
Estonian	Bosutiniib	Kroonilise müeloidse leukeemia ravi
Finnish	Bosutinibi	Kroonisen myeloosien leukemian hoito
French	Bosutinib	Traitement de la leucémie myéloïde chronique
German	Bosutinib	Behandlung der chronischen myeloischen Leukämie
Greek	Bosutinib	Θεραπεία της χρόνιας μυελοειδούς λευχαιμίας
Hungarian	Bozutinib	Krónikus myeloid leukémia kezelése
Italian	Bosutinib	Trattamento della leucemia mieloide cronica
Latvian	Bosutinibs	Hroniskas mieloleikozes ārstēšana
Lithuanian	Bozutinibas	Lėtinės mielocitinės leukemijos gydymas
Maltese	Bosutinib	Kura tal-lewkimja mjelojda kronika
Polish	Bosutynib	Leczenie przewlekłej białaczki szpikowej
Portuguese	Bosutinib	Tratamento da leucemia mielóide crónica
Romanian	Bosutinib	Tratamentul leucemiei mieloide cronice
Slovak	Bosutinib	Liečba chronickej myeloidnej leukémie
Slovenian	bosutinib	Zdravljenje kronične mieloične levkemije
Spanish	Bosutinib	Tratamiento de la leucemia mieloide crónica
Swedish	Bosutinib	Behandling av kronisk myeloid leukemi
Norwegian	Bosutinib	Behandling av kronisk myelogen leukemi
Icelandic	Bosutíníb	Meðferð við langvinnu kyrningahvítblæði

¹ At the time of designation