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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Bafetinib for the treatment of chronic myeloid leukaemia

First publication	22 June 2010
Rev.1: sponsor's change of address	13 March 2013
Rev.2: withdrawal from the Community Register	23 July 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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2013 on request of the Sponsor.

On 10 June 2010, orphan designation (EU/3/10/731) was granted by the European Commission to Eudax Srl, Italy, for bafetinib for the treatment of chronic myeloid leukaemia.

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.

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

At the time of designation, CML affected approximately 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 41,000 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 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 bafetinib 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?

Bafetinib is expected to work mainly by blocking a type of enzyme called BCR-ABL tyrosine kinase, as well as modified forms of this enzyme, which are found in most CML patients. The BCR-ABL tyrosine kinase is produced by leukaemia cells, and causes them to multiply uncontrollably. By blocking this enzyme, as well as other kinases, bafetinib is expected to control the spread of leukaemia cells.

What is the stage of development of this medicine?

The effects of bafetinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with bafetinib which included patients with CML were ongoing.

At the time of submission, bafetinib was not authorised anywhere in the EU for CML. Orphan designation of bafetinib had been granted in the United States of America for Philadelphia chromosome-positive (Ph+) CML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 February 2010 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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[1], Norwegian and Icelandic

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

[1] At the time of designation