4 February 2015
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Nilotinib for the treatment of chronic myeloid leukaemia

First publication	18 July 2007
Rev.1: information about Marketing Authorisation	30 November 2007
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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 22 May 2006, orphan designation (EU/3/06/375) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for nilotinib for the treatment of chronic myeloid leukaemia.

What is chronic myeloid leukaemia?

Chronic myeloid leukemia (CML) 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 leukemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukemias. In myeloid leukemia 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. The disease can develop very slowly, which is why it is called "chronic" myeloid leukemia. Chronic myeloid leukemia is life-threatening.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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

At the time of designation, chronic myeloid leukaemia affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 47,000 people*, and is below the ceiling 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 leukemia is complex and depends on a number of factors including the type of leukemia, the extent of the disease and whether the leukemia 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 leukemia 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 were used. Another product is also used which blocks (inhibits) growth signals within cancer cells and prevents a series of chemical reactions that cause the cell to grow and divide thus stopping cancer cells to grow. Bone marrow transplantation was also used.

Nilotinib could be of potential significant benefit for the treatment of chronic myeloid leukaemia mainly because it might improve the long-term outcome of the patients. 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?

Enzymes are proteins produced by the human body that speed up the transformation of certain substances into other substances. Nilotinib blocks (inhibits) a certain class of enzymes called tyrosine kinases. These enzymes play a role in a cascade of molecular reactions that bring a certain signal from outside the cell into the cell thereby controlling the growth of cells. In chronic myeloid leukaemia, the function of these enzymes is disturbed causing uncontrolled growth and multiplication of the cancer cells. Nilotinib might, by inhibition of one or more of these enzymes activity, at certain levels in the cascade, help in slowing down or stopping the further growth of the cancer cells.

What is the stage of development of this medicine?

The effects of nilotinib 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.

Nilotinib was not authorised anywhere worldwide for chronic myeloid leukaemia 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 April 2006 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.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

Update: Nilotinib (Tasigna) has been authorised in the EU since <date> for the treatment of adult patients with newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase.

More information on Tasigna 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

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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;
- 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:

Novartis Europharm Limited
Frimley Business Park
Camberley GU16 7SR
United Kingdom
Tel. +41 61 324 11 11 (Switzerland)
E-mail: orphan.enquiries@novartis.com

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

[1] At the time of designation