



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for treatment of chronic myeloid leukaemia

First publication	11 October 2005
Rev.1: information about Marketing Authorisation	29 November 2005
Rev.2: administrative update	28 February 2007
Rev.3: withdrawal from the Community Register	11 May 2012
Rev.4: administrative update	5 December 2013
Rev.5: sponsor's change of address	4 February 2015
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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in November 2011 at the end of the period of market exclusivity.

On 14 February 2001, orphan designation (EU/3/01/021) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of chronic myeloid leukaemia.

What is chronic myeloid leukaemia?

Chronic myeloid leukaemia is a disease in which cancer cells are found in the blood and 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. 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 0.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 34,000 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 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. Current treatment of chronic myeloid leukaemia is chemotherapy (using drugs to kill cancer cells) and immunotherapy (using drugs that stimulate the body's own immune system to kill cancer cells). Sometimes a combination of immunotherapy and chemotherapy may be used. Bone marrow transplantation is also used. Several treatments were authorised at the time of submission of the application for orphan drug designation.

Imatinib mesilate could be of potential significant benefit for the treatment of chronic myeloid leukaemia, because it may act in a different way to other drugs and it might improve the long-term outcome of patients.

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?

Enzymes are proteins produced by the human body that speed up the conversion of certain substances into other substances. Imatinib mesilate blocks (inhibits) the enzyme tyrosine kinase. This enzyme plays a role in a cascade of molecular reactions of bringing a certain external signal inside the cell itself, thereby controlling the growth of the cell. In cancer cells, the function of this enzyme is disturbed causing uncontrolled growth and multiplication of the cancer cells. Imatinib mesilate might, by inhibition of this enzyme activity, 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 imatinib mesilate 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.

Imatinib mesilate was not marketed or designated as orphan medicinal product elsewhere, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 December 2000 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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

Update: Imatinib mesilate (Glivec) has been authorised in the EU since 7 November 2001 for the treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment.

Glivec is also indicated for the treatment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis.

More information on Glivec 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](http://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](http://orphanet.eu), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](http://eurordis.eu), 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	Imatinib mesilate	Treatment of chronic myeloid leukaemia
Danish	Imatinib mesilat	Behandling af kronisk myeloid leukæmi
Dutch	Imatinib-mesilaat	Behandeling van chronische myeloïde leukemie
Finnish	Imatinibi mesilaatti	Kroonisen myelooisen leukaemian hoitoon
French	Imatinib mésilate	Traitement de la leucémie myéloïde chronique
German	Imatinib mesilat	Behandlung der chronisch-myeloischen Leukämie
Greek	Imatinib σουλφονικό μεθάνιο	Θεραπεία χρόνιας μυελοειδούς λευκαιμίας
Italian	Imatinib mesilato	Trattamento della leucemia mieloide cronica
Portuguese	Imatinib mesilato	Tratamento della leucemia mieloide cronica
Spanish	Imatinib mesilato	Tratamiento de la leucemia mieloide crónica
Swedish	Imatinib mesylat	Behandling av kronisk myeloisk leukemi

¹ At the time of designation