4 February 2015
EMA/COMP/275619/2005 Rev.4
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for the treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome

First publication	4 January 2006
Rev.1: information about Marketing Authorisation	28 February 2007
Rev.2: withdrawal from the Community Register	23 April 2012
Rev.3: administrative update	5 December 2013
Rev.4: 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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2012 on request of the sponsor.

On 28 October 2005, orphan designation (EU/3/05/320) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome.

What is chronic eosinophilic leukaemia and the hypereosinophilic syndrome?

Chronic eosinophilic leukaemia is a disease in which cancer cells are found in the blood, the bone marrow and in tissues. 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 chronic eosinophilic leukaemia blasts that are developing into white blood cells called eosinophils are affected. The eosinophils become too

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

numerous and are then found in the bone marrow, blood and in other tissues such as the heart, lungs, nerves and skin. The excess of eosinophils in tissues can cause disturbances in the function and thereby damage the affected organs. In many cases, it is impossible to find the origin of the cancer cells and then the disease is called the hypereosinophilic syndrome. The disease can develop very slowly, which is why it is called chronic. Chronic eosinophilic leukaemia and the hypereosinophilic syndrome are life-threatening.

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

At the time of designation, chronic eosinophilic leukaemia and the hypereosinophilic syndrome affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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?

No satisfactory methods exist that were authorised at the time of application.

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 to bring a certain signal from outside the cell into the cell thereby controlling the growth of the cells. 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?

At the time of submission of the application for orphan designation, clinical trials in patients with chronic eosinophilic leukaemia and the hypereosinophilic syndrome were ongoing.

Imatinib mesilate was not marketed anywhere worldwide for chronic eosinophilic leukaemia and the hypereosinophilic syndrome, at the time of submission.

Orphan designation of imatinib mesilate was granted in Europe and in the United States for chronic myeloproliferative leukaemia and for gastrointestinal stromal tumours.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 August 2005 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 466,600,000 (Eurostat 2005).

Update: Imatinib mesilate (Glivec) has been authorised in the EU since 28 November 2006 for treatment of adult patients with hypereosinophilic syndrome (HES) and chronic eosinophilic leukaemia (CEL).

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

_____

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	Imatinib mesilate	Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome
Czech	Imatinib mesilát	Léčba chronické eozinofilní leukemie / hypereozinofilního syndromu
Danish	Imatinib mesilat	Behandling af kronisk eosinofil leukæmi og hypereosinofilt syndrom
Dutch	Imatinib mesilaat	Behandeling van chronische eosinofiele leukemie en het hypereosinofiel syndroom
Estonian	Imatiniibmesülaat	Kroonilise eosinofiilse leukeemia ja hüpereosinofiilse sündroomi ravi
Finnish	Imatinibi mesilaatti	Kroonisen eosinofiilisen leukemian ja hypereosinofiilisen oireyhtymän hoito
French	Imatinib mésylate	Traitement de la leucémie chronique à éosinophiles et du syndrome hyperéosinophilique
German	Imatinib mesilat	Behandlung der eosinophile Leukämie und des hypereosinophilen Syndroms
Greek	Imatinib mesilate	Θεραπεία της χρονικής ηωσινοφιλικής λευχαιμίας και του υπερηωσινοφιλικού συνδρόμου
Hungarian	Imatinib mezilát	Eosinophil leukaemia és hypereosinophil syndroma kezelése
Italian	Imatinib mesilato	Trattamento della leucemia eosinofila cronica e della sindrome ipereosinofila
Latvian	Imatiniba mezilāts	Hroniskas eozinofīlas leikēmija un hipereozinofīlā sindroma ārstēšana
Lithuanian	Imatinibo mezilatas	Lėtinės eozinofilinės leukemijos ir hipereozinofilinio sindromo gydymas
Polish	Imatynib w postaci metanosulfonianu	Leczenie przewlekłej białaczki eozynofilowej i zespołu hipereozynofilowego
Portuguese	Mesilato de imatinib	Tratamento da leucemia eosinófila crónica e do síndrome hipereosinofílico
Slovak	Imatinibi mesilas	Liečba chronickej eozinofilnej leukémie a hypereozinofilného syndrómu
Slovenian	Imatinib mesilat	Zdravljenje kronične eozinofilne levkemije in hipereozinofilnega sindroma
Spanish	Imatinib mesilato	Tratamiento de la leucemia eosinofílica crónica y del síndrome hipereosinofílico
Swedish	Imatinib mesylat	Behandling av kronisk eosinofil leukemi och hypereosinofilt syndrom
Norwegian	Imatinib mesilat	Behandling av kronisk eosinofil leukemi og hypereosinofilt syndrom

[1] At the time of designation

Language	Active ingredient	Indication
Icelandic	Ímatíníb mesílat	Meðferð við rauðkyrningahvítblæði og rauðkyrningaoffjölgunar heilkenni af óþekktri orsök