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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for the treatment of mastocytosis

First publication	29 November 2005
Rev.1: withdrawal from the Community Register	30 November 2007
Rev.2: 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Please note that this produc twas withdrawn from the Community Register of designated Orphan Medicinal Products in November 2007 on request of the sponsor.

On 26 August 2005, orphan designation (EU/3/05/306) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of mastocytosis.

What is mastocytosis?

Mastocytosis is a disease in which a certain type of cells (so-called mast cells) accumulate excessively in the bone marrow and in other organs. 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 mastocytosis develops, large numbers of a certain white blood cell called mast cells are produced. In normal circumstances these cells are mainly located in the skin and in the linings of the intestine. Their role is two-fold: they take part in the defence of these tissues against diseases and they contribute in the development of allergic reactions.

The manifestation of mastocytosis is variable. In most of the patients, mainly in children, only the skin is involved and these lesions may spontaneously disappear (cutaneous mastocytosis). In some patients, more often occuring in adults, the cancer cells become aggressive tumours infiltrating organs (systemic mastocytosis) leading to organ failure and poor long-term outcome.

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, mastocytosis 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 mastocytosis were ongoing.

Imatinib mesilate was not marketed anywhere worldwide for mastocytosis, 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 13 July 2005 recommending the granting of this designation.

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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.

*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).

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 mastocytosis
Czech	Imatinib mesilát	Léčba mastocytózy
Danish	Imatinib mesilat	Behandling af mastocytose
Dutch	Imatinib mesilaat	Behandeling van mastocytose
Estonian	Imatiniibmesülaat	Mastotsütoosi ravi
Finnish	Imatinibi mesilaatti	Mastosytoosin hoito
French	mésylate d'Imatinib	Traitement de la mastocytose
German	Imatinib mesilat	Behandlung der Mastozytose
Greek	Imatinib mesilate	Θεραπεία της ιστιοκυττάρωσης
Hungarian	Imatinib mezilát	Mastocytosis kezelése
Italian	Imatinib mesilato	Trattamento della mastocitosi
Latvian	Imatiniba mezilāts	Mastocitozes ārstēšana
Lithuanian	Imatinibo mezilatas	Mastocitozės gydymas
Polish	Imatynib w postaci metanosulfonianu	Leczenie mastocytozy
Portuguese	Mesilato de imatinib	Tratamento da mastocitose
Slovak	Imatinibi mesilas	Liečba mastocytózy
Slovenian	Imatinib mesilat	Zdravljenje dermatofibrosarkoma protuberans
Spanish	Imatinib mesilato	Tratamiento de la mastocitosis
Swedish	Imatinib mesylat	Bahandling av mastocytos
Norwegian	Imatinib mesilat	Behandling av mastocytose
Icelandic	Imatíníb mesílat	Meðferð mastocytosis

[1] At the time of designation