4 February 2015
EMA/COMP/386759/2005 Rev.4
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for the treatment of myelodysplastic / myeloproliferative diseases

First publication	8 August 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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2012 on request of the sponsor.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 23 December 2005, orphan designation (EU/3/05/340) was granted by the European Commission Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of myelodysplastic / myeloproliferative diseases.

What are myelodysplastic / myeloproliferative diseases?

Myelodysplastic / myeloproliferative diseases (MDS/MPD) are a distinct group of disorders 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 the disease develops, the bone marrow produces large amounts of one or several types of abnormal blood cells. Even when mature, because of their abnormality, these blood cells are not able to exercise their normal function and will lead to a variety of symptoms such as fatigue or weakness (due to anaemia, the red cells

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deficit), infections (due to decrease in normal white blood cells) or easy bruising or abnormal bleeding (platelet deficit). Various organs can be infiltrated by these cells and can, apart from increased size, start to malfunction because of this. Myelodysplastic / myeloproliferative diseases (MDS/MPD) are life-threatening.

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

At the time of designation, myelodysplastic / myeloproliferative diseases affected less than 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 75,000 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 MDS/MPD is complex and depends on a number of factors including the type and the extent of the disease and whether this has been treated before. It also depends on the patient's age, symptoms, and general health. Current treatments for MDS/MPD include bone marrow transplantation and chemotherapy (using drugs to kill cancer cells). Some anticancer agents were authorised in the Community for the treatment of MDS/MPD at the time of submission of the application for orphan designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that imatinib mesilate might be of potential significant benefit for the treatment of MDS/MPD, because it may act in a different way than the available methods and it might improve the long-term outcome of the 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 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 myelodysplastic / myeloproliferative diseases were ongoing.

Imatinib mesilate was not marketed anywhere worldwide for myelodysplastic / myeloproliferative diseases, 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.

[*]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).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2005 recommending the granting of this designation.

Update: Imatinib mesilate (Glivec) has been authorised in the EU since 28 November 2006 for treatment of adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with PDGFR gene re-arrangements.

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

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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:

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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	Imatinib mesilate	Treatment of myelodysplastic / myeloproliferative diseases
Czech	Imatinib mesilát	Myelodysplastického / myeloproliferativnhoí onemocnění
Danish	Imatinib mesilat	Behandling af myelodysplastiske / myeloproliferative sygdomme
Dutch	Imatinib mesilaat	Behandeling van myelodysplastische / myeloproliferatieve aandoeningen
Estonian	Imatiniibmesülaat	Müelodüsplastiliste/ müeloproliferatiivsete haiguste ravi
Finnish	Imatinibi mesilaatti	Myelodysplastisten / myeloproliferatiivisten sairauksien hoito
French	Imatinib mésylate	Traitement des syndromes myéloprolifératifs / myélodysplasiques
German	Imatinib mesilat	Behandlung myelodysplastischer / myeloproliferativer Erkrankungen
Greek	Imatinib mesilate	Θεραπεία μυελοδυσπλαστικών / μυελοϋπερπλαστικών νόσων
Hungarian	Imatinib mezilát	Myelodysplasias / myeloproliferatív betegségek kezelése
Italian	Imatinib mesilato	Trattamento delle sindromi mielodisplastiche / malattie mieloproliferative
Latvian	Imatiniba mezilāts	Mielodisplastisku / mieloproliferatīvu slimību ārstēšanai
Lithuanian	Imatinibo mezilatas	Mielodisplastinės / mieloproliferacinės ligos gydymas
Polish	Imatynib w postaci metanosulfonianu	Leczenie chorób mieloproliferacyjnych i mielodysplastycznych
Portuguese	Mesilato de imatinib	Tratamento de doenças mielodisplásicas / mieloproliferativas
Slovak	Imatinibi mesilas	Liečba myelodysplastických / myeloproliferatívnych ochorení
Slovenian	Imatinib mesilat	Zdravljenje mielodisplastičnega sindroma / mieloproliferativne bolezni
Spanish	Imatinib mesilato	Tratamiento de las enfermedades mielodisplásicas / mieloproliferativas
Swedish	Imatinib mesylat	Behandling av myelodysplastiska / myeloproliferativa sjukdomar
Norwegian	Imatinib mesilat	Behandling av myelodysplastiske / myeloproliferative sykdommerh
Icelandic	Imatíníb mesílat	Mergmisþroska-/ mergfrumnafjölgunar sjúkdómar

[1] At the time of designation