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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for the treatment of dermatofibrosarcoma protuberans

First publication	29 November 2005
Rev.1: information about Marketing Authorisation	28 February 2007
Rev.2: withdrawal from the Community Register	19 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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2012 on request of the sponsor.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 26 August 2005, orphan designation (EU/3/05/305) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of dermatofibrosarcoma protuberans.

What is dermatofibrosarcoma protuberans?

Dermatofibrosarcoma is a relatively slowly growing skin cancer, which can occur anywhere in the body but most of them occur on the trunk or on the arms and legs. The pattern of growth is usually slow and persistent, and as the lesion enlarges over many years, it becomes a knob (protuberant). The cancer consists of one or several hard small lumps that are usually covered by a dark red-blue skin, which tends to be fixed to the lumps. As it grows slowly it is often ignored until it grows large. This type of skin cancer has the potential to infiltrate in the local surrounding tissue but will only rarely spread to other parts of the body. Because of its local aggressive nature, surgical excision is often extensive and can include underlying tissues such as muscles and bone. This may lead to severe restrictions of the use of that part of the body. Despite extensive surgery, the cancer often comes

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back. Dermatofibrosarcoma protuberans is chronically debilitating and in some cases can be life threatening due to extensive invasion.

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

At the time of designation, dermatofibrosarcoma protuberans 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 medicinal products were authorised for the treatment of dermatofibrosarcoma protuberans in the Community at the time of submission of the application for orphan drug designation. The treatment option is limited to various surgical procedures.

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 dermatofibrosarcoma protuberans were ongoing.

Imatinib mesilate was not marketed anywhere worldwide for dermatofibrosarcoma protuberans, 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.

Update: Imatinib mesilate (Glivec) has been authorised in the EU since 13 September 2006 for the treatment of adult patients with unresectable dermafibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not elegible for surgery.

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

*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).

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 dermatofibrosarcoma protuberans
Czech	Imatinib mesilát	Léčba dermatofibrosarkomu protuberans
Danish	Imatinib mesilat	Behandling af dermatofibrosarcoma protuberans
Dutch	Imatinib mesilaat	Behandeling van dermatofibrosarcoma protuberans
Estonian	Imatiniibmesülaat	Protuberantse dermatofibrosarkoomi ravi
Finnish	Imatinibi mesilaatti	Dermatofibrosarkooma protuberanssin hoito
French	Imatinib mésylate	Traitement du dermatofibrosarcome protubérant
German	Imatinib mesilat	Behandlung des Dermatofibrosarkoma protuberans
Greek	Imatinib mesilate	Θεραπεία του προέχοντος Δερματοϊνοσαρκώματος
Hungarian	Imatinib mezilát	Protuberans dermatofibrosarcoma kezelése
Italian	Imatinib mesilato	Trattamento del dermatofibrosarcoma protuberans
Latvian	Imatiniba mezilāts	Paugurainās dermatofibrosarkomas ārstēšana
Lithuanian	Imatinibo mezilatas	Iškiliosios dermatofibrosarkomos gydymas
Polish	Imatynib w postaci metanosulfonianu	Leczenie włókniakomięsaka guzkowatego skóry
Portuguese	Mesilato de imatinib	Tratamento do dermatofibrosarcoma protuberante
Slovak	Imatinibi mesilas	Liečba dermatofibrosarkómu protuberans
Slovenian	Imatinib mesilat	Zdravljenje akutne limfoblastne levkemije
Spanish	Imatinib mesilato	Tratamineto del dermatofibrosarcoma protuberans
Swedish	Imatinib mesylat	Behandling av dermatofibrosarkom protuberans
Norwegian	Imatinib mesilat	Behandling av dermatofibrosarcoma protuberans
Icelandic	Imatíníb mesílat	Meðferð dermatofibrosarcoma protuberans

[1] At the time of designation