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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib mesilate for the treatment of malignant gastrointestinal stromal tumours

First publication	11 October 2005
Rev.1: administrative update	29 November 2005
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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2012 on request of the sponsor.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 20 November 2001, orphan designation (EU/3/01/061) was granted by the European Commission Novartis Europharm Limited, United Kingdom, for imatinib mesilate for the treatment of malignant gastrointestinal stromal tumours.

What are malignant gastrointestinal stromal tumours?

Malignant gastrointestinal stromal tumours are the most common subtype of gastro-intestinal sarcomas, which belong to the group of soft tissue sarcomas. Soft tissue sarcomas are cancers of the tissues that support, surround and protect the organs of the body, such as muscle tissue and fat tissue. The cause of gastrointestinal stromal tumours is largely unknown. Gastrointestinal stromal tumours are most common in the stomach (60-70%), followed by small intestine (20-30%), and colon and rectum (5%). Gastrointestinal stromal tumours occur predominantly in middle-aged and older persons and are considered as a life-threatening condition.

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

At the time of designation, malignant gastrointestinal stromal tumours affected approximately 0.06 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,300 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?

At the time of submission of the application for orphan drug designation the method of treatment was surgery. There were no medicinal products authorised for the condition in the Community at the time of submission.

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 responsible of bringing a certain external signal inside the cell, thereby controlling the growth of the cell itself. 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, one clinical trial in patients with malignant gastrointestinal stromal tumours was ongoing.

Imatinib mesilate was not marketed anywhere worldwide for the treatment of malignant gastrointestinal stromal tumours or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2001 recommending the granting of this designation.

Update: Imatinib mesilate (Glivec) was authorised in the EU on 24 May 2002 for the treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST).

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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

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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United Kingdom
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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 malignant gastrointestinal stromal tumours
Danish	Imatinib mesilat	Behandling af maligne gastrointestinale stromale tumorer
Dutch	Imatinib mesilaat	Behandeling van maligne gastro-intestinale stromale tumoren
Finnish	Imatinibi mesilaatti	Mahalaukun tai suoliston pahanlaatuisten stroomakasvainten (GIST) hoito
French	Imatinib mésylate	Traitement des tumeurs malignes stromales gastro-intestinales
German	Imatinib mesilat	Behandlung von malignen gastrointestinalen Stromatumoren
Greek	Σουλφονικό μεθάνιο	Θεραπεία των κακοήθων στρωματικών γαστρεντερικών όγκων
Italian	Imatinib mesilato	Trattamento dei tumori stromali maligno del tratto gastro-intestinale
Portuguese	Mesilato de imatinib	Tratamento de tumores maligno do estroma gastrintestinal
Spanish	Mesilato de Imatinib	Tratamiento de los tumores malignos del estroma gastrointestinal
Swedish	Imatinib mesylat	Behandling av maligna gastrointestinala stromacellstumörer

[1] At the time of designation