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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Midostaurin for the treatment of mastocytosis

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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.	

On 4 August 2010, orphan designation (EU/3/10/765) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for midostaurin for the treatment of mastocytosis.

What is mastocytosis?

Mastocytosis is a disorder in which there are too many mast cells in various organs in the body. Mast cells are a type of white blood cells that originate in the bone marrow and then migrate to other parts of the body such as the skin and intestine, where they take part in defending against infections and contribute to the development of allergic reactions by releasing a number of substances including histamine.

The symptoms of mastocytosis vary from patient to patient. In children, usually this disorder only affects the skin ('cutaneous mastocytosis') and causes a red and itchy rash, which may disappear on its own. In some patients, mainly adults, the disorder progresses into 'systemic mastocytosis', in which the mast cells become aggressive tumours that infiltrate organs, such as the intestine, the liver, the spleen and the bone marrow. This causes various symptoms such as palpitations and fainting, bone pain, tiredness, weight loss, diarrhoea, nausea (feeling sick), vomiting and stomach ache.

Mastocytosis is a condition that is debilitating in the long term and may be life threatening in those patients who develop the systemic form of the disorder.

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

At the time of designation, mastocytosis affected approximately 0.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 46,000 people*, and is below the threshold 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 designation, only treatments aimed at relieving the symptoms of mastocytosis were available. They included antihistamines to block the action of histamine produced by the mast cells.

The sponsor has provided sufficient information to show that midostaurin might be of significant benefit for patients with mastocytosis because early studies indicate that it might slow down the progression of the disorder. 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?

Midostaurin is expected to work by blocking types of enzymes known as tyrosine kinases. These enzymes can be found in some receptors on the surface of mast cells, including the 'KIT' receptors, which are involved in stimulating the growth of mast cells in mastocytosis. By blocking these receptors, midostaurin is expected to control the growth of mast cells, slowing down the progression of mastocytosis.

What is the stage of development of this medicine?

The effects of midostaurin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with midostaurin in patients with mastocytosis were ongoing.

At the time of submission, midostaurin was not authorised anywhere in the EU for mastocytosis. Orphan designation of midostaurin had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 May 2010 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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	Midostaurin	Treatment of mastocytosis
Bulgarian	Мидостаурин	Лечение на мастоцитоза
Czech	Midostaurin	Léčba mastocytózy
Danish	Midostaurin	Behandling af mastocytose
Dutch	Midostaurine	Behandeling van mastocytose
Estonian	Midostaurin	Mastotsütoosi ravi
Finnish	Midostauriini	Mastosytoosin hoito
French	Midostaurine	Traitement de la mastocytose
German	Midostaurin	Behandlung der Mastozytose
Greek	Midostaurin	Θεραπεία της μαστοκυττάρωσης
Hungarian	Midostaurin	Mastocytosis kezelése
Italian	Midostaurina	Trattamento della mastocitosi
Latvian	Midostaurīns	Mastocitozes ārstēšana
Lithuanian	Midostaurinas	Mastocitozės gydymas
Maltese	Midostaurin	Kura tal-mastoċitosi
Polish	Midostauryna	Leczenie mastocytozy
Portuguese	Midostaurina	Tratamento da mastocitose
Romanian	Midostaurină	Tratamentul mastocitozei
Slovak	Midostaurín	Liečba mastocytózy
Slovenian	Midostavrin	Zdravljenje dermatofibrosarkoma protuberans
Spanish	Midostaurina	Tratamiento de la mastocitosis
Swedish	Midostaurin	Bahandling av mastocytos
Norwegian	Midostaurin	Behandling av mastocytose
Icelandic	Mídóstaurín	Meðferð mastócýtosis

[1] At the time of designation