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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Midostaurin for the treatment of acute myeloid leukaemia

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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 29 July 2004, orphan designation (EU/3/04/214) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for midostaurin for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia is a disease in which cancer cells are found in the blood and 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 leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukaemias. In myeloid leukaemia blasts that are developing into white blood cells called granulocytes are affected. The blasts do not mature and become too many. These blast cells are then found in the blood and also accumulate in the bone marrow. Leukaemia can be acute (when it develops quickly with many blasts). Acute myeloid leukaemia is life-threatening.

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

At the time of designation, acute myeloid leukaemia affected approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 32,000 people*, and is below the ceiling

*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 464,200,000 (Eurostat 2004).

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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 leukaemia is complex and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. The primary treatment of acute myeloid leukaemia is chemotherapy (using drugs to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Midostaurin might be of potential significant benefit for the treatment of acute myeloid leukaemia, because it may act in a different way than other medicines and it might add to the effect of the current standard treatment. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary 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. Midostaurin inhibits an enzyme, the so-called fms-like tyrosine kinase 3 (FLT3). It has been found that this enzyme plays an important role in the survival and self-renewal of the blast cells, but not anymore after the maturation of these cells. FLT3 has been identified in >80% of the myeloid leukaemia blast cells. Midostaurin might, by inhibition of this FLT3 enzyme activity, help in slowing down or stopping the further growth of the leukaemia cells and might help in the destruction of these cells carrying FLT3.

What is the stage of development of this medicine?

The effects of midostaurin were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with acute myeloid leukaemia were ongoing.

Midostaurin was not marketed anywhere worldwide for acute myeloid leukaemia 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 16 June 2004 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.

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 acute myeloid leukaemia
Czech	Midostaurin	Léčba akutní myeloidní leukémie
Danish	Midostaurin	Behandling af akut myeloid leukæmi
Dutch	Midostaurine	Behandeling van acute myeloïde leukemie
Estonian	Midostaurin	Akuutse müeloidse leukeemia ravi.
Finnish	Midostauriini	Akuutin myelooisen leukaemian hoito
French	Midostaurine	Traitement de la leucémie myéloïde aiguë
German	Midostaurin	Behandlung der akuten myeloischen Leukämie
Greek	Midostaurin	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Midostaurin	Akut myeloid leukaemia kezelése
Italian	Midostaurina	Trattamento della leucemia mieloide acuta
Latvian	Midostaurīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Midostaurinas	Ūminės mieloleukozės gydymas
Maltese	Midostaurin	Treatment of acute myeloid leukaemia
Polish	Midostauryin	Leczenie ostrej białaczki szpikowej
Portuguese	Midostaurina	Tratamento da leucemia mielóide aguda
Slovak	Midostaurín	Liečba akútnej myeloickej leukémie
Slovenian	Midostavrin	Zdravljenje akutne mieloične levkemije
Spanish	Midostaurina	Tratamiento de la leucemia mieloide aguda
Swedish	Midostaurin	Behandling av akut myeloisk leukemi
Norwegian	Midostaurin	Behandling av akutt myelogen leukemi
Icelandic	Midostaurin	Til meðferðar við bráðu kyrningahvítblæði

[1] At the time of designation