17 April 2015
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lestaurtinib for the acute myeloid leukaemia

First publication	23 February 2009
Rev.1: administrative update	25 May 2011
Rev.2: sponsor's name and address change	21 March 2014
Rev.3: withdrawal from the Community Register	17 April 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2015 on request of the Sponsor.

On 15 July 2006, orphan designation (EU/3/06/389) was granted by the European Commission to Cephalon UK Ltd, United Kingdom, for lestaurtinib for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Cephalon Europe, France, in May 2007. Cephalon Europe subsequently became Teva Santé.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells. In this disease, the bone marrow produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is life-threatening because these immature cells take the place of the normal white blood cells. As a result, the patient's ability to fight diseases is reduced.

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

At the time of designation, acute myeloid leukaemia affected approximately 0.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 42,000 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 AML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. The primary treatment for AML is chemotherapy (using medicines to kill cancer cells).

Satisfactory argumentation has been submitted by the sponsor to justify that lestaurtinib could be of potential significant benefit for the treatment of acute myeloid leukaemia because it could improve the overall outcome of the patients. 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 transformation of certain substances into other substances. Lestaurtinib blocks (inhibits) a certain class of enzymes called tyrosine kinases. These enzymes play 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 cells. In AML, the function of some of these enzymes is disturbed causing uncontrolled growth and multiplication of the cancer cells. By inhibiting this enzyme activity, lestaurtinib might 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 lestaurtinib were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with treatment of acute myeloid leukaemia were ongoing.

Lestaurtinib was not authorised anywhere worldwide for treatment of acute myeloid leukaemia, at the time of submission. Orphan designation of lestaurtinib was granted in the United States for treatment of acute myeloid leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 June 2006 recommending the granting of this designation.

[*]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 468,900,000 (Eurostat 2006).

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

[1] At the time of designation