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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Homoharringtonine for the treatment of acute myeloid leukemia

On 20 October 2004, orphan designation (EU/3/04/228) was granted by the European Commission to Stragen France SAS, for homoharringtonine for the treatment of acute myeloid leukemia.

The sponsorship was transferred to ChemGenex Europe SAS, France, in January 2009 and subsequently to Teva Pharma GmbH, Germany, in December 2012.

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 normally 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 32,000 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).

*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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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 leukemia 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.

Homoharringtonine could be of potential significant benefit for the treatment of acute myeloid leukaemia because it may act in a different way than the already authorised drugs. This 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?

Homoharringtonine (HHT) is a substance that was originally isolated from the entire plant of the evergreen tree *Cephalotaxus harringtonia K. Koch van harringtonia* present in China. Homoharringtonine belongs to a group of medicines called alkylating agents. Alkylating agents are highly reactive chemicals that bind to some components of the cell and consequently damage or kill the cells, in particular those that are proliferating. It is thought that through this mechanism, homoharringtonine might stop the growth of the cancer cells.

What is the stage of development of this medicine?

The effects of homoharringtonine were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with acute myeloid leukemia were completed.

Homoharringtonine was not marketed anywhere worldwide for acute myeloid leukemia, at the time of submission. Orphan designation of homoharringtonine was granted in the United States for treatment of chronic myelogenous leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 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	Homoharringtonine	Treatment of acute myeloid leukaemia
Bulgarian	Хомохарингтонин	Лечение на остра миелоидна левкемия
Czech	Homoharringtonin	Léčba akutní myeloidní leukémie
Danish	Homoharringtonin	Behandling af akut myeloid leukæmi
Dutch	Homoharringtonine	Behandeling van acute myeloïde leukemie
Estonian	Homoharringtoniin	Akuutse müeloidse leukeemia ravi.
Finnish	Homoharringtoniini	Akuutin myelooisen leukemian hoito
French	Homoharringtonine	Traitement de la leucémie myéloïde aiguë
German	Homoharringtonin	Behandlung der akuten myeloischen Leukämie
Greek	Homoharringtonine	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Homoharringtonin	Akut myeloid leukaemia kezelése
Italian	Omoharringtonina	Trattamento della leucemia mieloide acuta
Latvian	Homoharingtonīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Homoharingtoninas	Ūminės mieloleukozės gydymas
Maltese	Homoharringtonine	Kura tal-lewkimja mjelojda akuta
Polish	Homoharringtonina	Leczenie ostrej białaczki szpikowej
Portuguese	Homoharringtonina	Tratamento da leucemia mielóide aguda
Romanian	Homoaringtonină	Tratamentul leucemiei mieloide acute
Slovak	Homoharringtonín	Liečba akútnej myeloickej leukémie
Slovenian	Homoharringtonin	Zdravljenje akutne mieloične levkemije
Spanish	Homoharringtonina	Tratamiento de la leucemia mieloide aguda
Swedish	Homoharringtonin	Behandling av akut myeloisk leukemi
Norwegian	Homoharringtonin	Behandling av akutt myelogen leukemi
Icelandic	Homoharringtonín	Til meðferðar við bráðu kyrningahvítblæði

[1] At the time of transfer of sponsorship