5 March 2013
EMA/COMP/44012/2004 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Homoharringtonine for treatment of chronic myeloid leukaemia

On 2 September 2004, orphan designation (EU/3/04/224) was granted by the European Commission to Stragen France SAS, France, for homoharringtonine for the treatment of chronic myeloid leukaemia.

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

What is chronic myeloid leukaemia?

Chronic myeloid leukaemia is a disease in which one specific type of abnormal blood cells multiplies abnormally in 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 multiply without any control. These blast cells are then found in the blood and also accumulate in the bone marrow. When this disease develops very slowly, it is called “chronic” myeloid leukaemia. Chronic myeloid leukaemia is life-threatening.

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

At the time of designation, chronic 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).

*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. Currently authorised treatments of chronic myeloid leukaemia include chemotherapy agents (using drugs to kill cancer cells) and immunotherapy agents (using drugs that stimulate the body's own immune system to kill cancer cells). Furthermore another product with another mechanism of action, has been recently designated as orphan and authorised in Europe. It works by blocking (inhibiting) growth signals within cancer cells and preventing a series of chemical reactions that cause the cell to grow and divide, the final result being that cancer cells stop growing. Sometimes a combination of immunotherapy and chemotherapies may be used. Bone marrow transplantation is also used. In conclusion, several treatments had been authorised for this indication at the time of submission of the application for orphan designation.

Homoharringtonine could be of potential significant benefit for the treatment of chronic myeloid leukaemia, because it may act in a different way than other already approved drugs and it might improve the long-term outcome of patients. 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 can damage or kill the cells. It is thought that through this mechanism, homoharringtonine might stop the cancer cells to grow.

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 chronic myeloid leukaemia were ongoing.

Homoharringtonine was not marketed or designated as orphan medicinal product elsewhere, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 22 July 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 chronic myeloid leukemia
Bulgarian	Хомохарингтонин	Лечение на хронична миелоидна левкемия
Czech	Homoharringtonin	Léčba chronické myeloidní leukémie
Danish	Homoharringtonin	Behandling af kronisk myeloid leukæmi
Dutch	Homoharringtonine	Behandeling van chronische myeloïde leukemie
Estonian	Homoharringtoniin	Kroonilise müeloidse leukeemia ravi
Finnish	Homoharringtoniini	Kroonisen myelooisen leukemian hoito
French	Homoharringtonine	Traitement de la leucémie myéloïde chronique
German	Homoharringtonin	Behandlung der chronischen myeloischen Leukämie
Greek	Homoharringtonine	Θεραπεία της χρόνιας μυελοειδούς λευχαιμίας
Hungarian	Homoharringtonin	Krónikus myeloid leukımia kezelıse
Italian	Omoharringtonina	Trattamento della leucemia mieloide cronica
Latvian	Homoharingtonīns	Hroniskas mieloleikozes ārstēšana
Lithuanian	Homoharingtoninas	Lėtinės mielocitinės leukemijos gydymas
Maltese	Homoharringtonine	Kura tal-lewkimja mjelojda kronika
Polish	Homoharringtonina	Leczenie przewlekłej białaczki szpikowej
Portuguese	Homoharringtonina	Tratamento da leucemia mielóide crónica
Romanian	Homoaringtonină	Tratamentul leucemiei mieloide cronice
Slovak	Homoharringtonín	Liečba chronickej myeloidnej leukémie
Slovenian	Homoharringtonin	Zdravljenje kronične mieloične levkemije
Spanish	Homoharringtonina	Tratamiento de la leucemia mieloide crónica
Swedish	Homoharringtonin	Behandling av kronisk myeloid leukemi
Norwegian	Homoharringtonin	Behandling av kronisk myelogen leukemi
Icelandic	Homoharringtonín	Meðferð við langvinnu hvítblæði í beinmerg

[1] At the time of transfer of sponsorship