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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Vosaroxin for the treatment of acute myeloid leukaemia

On 26 April 2012, orphan designation (EU/3/12/990) was granted by the European Commission to Sunesis Europe Ltd, United Kingdom, for vosaroxin for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are made) produces large numbers of abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood. Over a period of time, the abnormal cells replace the normal white blood cells, red blood cells and platelets (components that help the blood to clot) in the bone marrow.

AML is a long-term debilitating and life-threatening disease because it reduces the patient's ability to fight infections and causes bleeding in the gut and brain.

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

At the time of designation, AML affected approximately 0.8 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 41,000 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. At the time of designation, the main treatments for AML were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

*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. This represents a population of 506,300,000 (Eurostat 2011).

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The sponsor has provided sufficient information to show that vosaroxin might be of significant benefit for patients with AML because it works in a different way to existing treatments and early studies show that it might improve the outcome of patients when used in combination with other treatments such as chemotherapy. 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?

Vosaroxin is a cytotoxic (cell-killing) substance. It blocks an enzyme called topoisomerase II, which is involved in the division of DNA. When the enzyme is blocked, the DNA strands break, and this prevents the cancer cells from dividing, causing them to die.

What is the stage of development of this medicine?

The effects of vosaroxin have been evaluated in experimental models.

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

At the time of submission, vosaroxin was not authorised anywhere in the EU for AML. Orphan designation of vosaroxin had been granted in United States of America for the treatment of AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 March 2012 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	Vosaroxin	Treatment of acute myeloid leukaemia
Bulgarian	Возароксин	Лечение на остра миелоидна левкемия
Czech	Vosaroxin	Léčba akutní myeloidní leukémie
Danish	Vosaroxin	Behandling af akut myeloid leukæmi
Dutch	Vosaroxin	Behandeling van acute myeloïde leukemie
Estonian	Vosaroksiin	Akuutse müeloidse leukeemia ravi
Finnish	Vosaroksiini	Akuutin myelooisen leukemian hoito
French	Vosaroxine	Traitement de la leucémie aiguë myéloïde
German	Vosaroxin	Behandlung der akuten myeloischen Leukämie
Greek	Βοσαροξίνη	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Vosaroxin	Akut myeloid leukaemia kezelése
Italian	Vosaroxin	Trattamento della leucemia mieloide acuta
Latvian	Vozaroksīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Vosaroksinas	Ūmios mieloleukozės gydymas
Maltese	Vosaroxin	Kura tal-lewkimja mjelojda akuta
Polish	Wozaroksyna	Leczenie ostrej białaczki szpikowej
Portuguese	Vosaroxina	Tratamento da leucémia mielóide aguda
Romanian	Vosaroxin	Tratamentul leucemiei mieloide acute
Slovak	Vosaroxín	Liečba akútnej myeloickej leukémie
Slovenian	Vozaroksin	Zdravljenje akutne mieloične levkemije
Spanish	Vosaroxina	Tratamiento de la leucemia mieloide aguda
Swedish	Vosaroxin	Behandling av akut myeloisk leukemi
Norwegian	Vosaroksin	Behandling av akutt myelogen leukemi
Icelandic	Vósaroxín	Meðferð á bráðu kyrningahvítblæði

[1] At the time of designation