

20 December 2011
EMA/COMP/862754/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Resminostat for the treatment of Hodgkin's lymphoma

On 9 December 2011, orphan designation (EU/3/11/930) was granted by the European Commission to 4SC AG, Germany, for resminostat for the treatment of Hodgkin's lymphoma.

What is Hodgkin's lymphoma?

Hodgkin's lymphoma is a type of cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. Because lymph nodes are found throughout the body, the cancer can begin in almost any part of the body. In Hodgkin's lymphoma, white blood cells in the lymphatic system multiply too quickly and live for too long. These cancer cells can spread through the lymphatic system to other lymph nodes or through the bloodstream to other organs such as the spleen, where they can form new tumours.

Many people with Hodgkin's lymphoma can be cured if the disease is found and treated early. However, despite the treatments available, Hodgkin's lymphoma remains a serious and life-threatening disease, mainly because it is associated with poor survival in patients whose disease does not respond to treatment or has come back after previous treatment.

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

At the time of designation, Hodgkin's lymphoma was estimated to affect between 1 and 4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 51,000 and 203,000 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?

At the time of designation, several medicines were authorised for the treatment of Hodgkin's lymphoma in the EU. The main treatments for Hodgkin's lymphoma included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem

*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).

cell transplantation was also used when the disease had not responded to treatment or had come back after treatment. This is a complex procedure where patients receive their own stem cells to help restore the bone marrow.

The sponsor has provided sufficient information to show that resminostat might be of significant benefit for patients with Hodgkin's lymphoma because early studies show that it might improve the treatment of patients, particularly patients whose disease does not respond to treatment or has come back after previous treatment. 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?

Resminostat blocks the activity of enzymes called histone deacetylases, which are involved in turning genes 'on' and 'off' within cells. In Hodgkin's lymphoma, resminostat is expected to switch 'on' the genes that suppress the division and growth of the tumour cells. This is expected to lead to a reduction in the growth and division of the cancer cells.

What is the stage of development of this medicine?

The effects of resminostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with resminostat in patients with Hodgkin's lymphoma were ongoing.

At the time of submission, resminostat was not authorised anywhere in the EU for Hodgkin's lymphoma. Orphan designation of resminostat had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2011 recommending the granting of this designation.

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:

4SC AG
Klopferspitz 19a
82152 Martinsried
Germany
Telephone: +49 89 700763 0
Telefax: +49 89 700763 29
E-mail: resminostat@4sc.com

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Resminostat	Treatment of Hodgkin's lymphoma
Bulgarian	Ресминоста́т	Лечение на лимфом на Хочкин
Czech	Resminostat	Léčba Hodgkinova lymfomu
Danish	Resminostat	Behandling af Hodgkin lymfom
Dutch	Resminostat	Behandeling van Hodgkin lymfoom
Estonian	Resminostat	Hodgkini lümfoomi ravi
Finnish	Resminostaatti	Hodgkinin lymfooman hoito
French	Resminostat	Traitement du lymphome de Hodgkin
German	Resminostat	Behandlung des Hodgkin-Lymphoms
Greek	Ρεσμινοστάτη	Θεραπεία του λεμφώματος Hodgkin
Hungarian	Resminostat	Hodgkin lymphoma kezelése
Italian	Resminostat	Trattamento del linfoma di Hodgkin
Latvian	Resminostats	Hodžkina limfomas ārstēšana
Lithuanian	Resminostatas	Hodžkino limfomos gydymas
Maltese	Resminostat	Kura tal-linfoma ta' Hodgkin
Polish	Resminostat	Leczenie chłoniaka Hodgkina (ziarnicy złośliwej)
Portuguese	Resminostat	Tratamento do linfoma de Hodgkin
Romanian	Resminostat	Tratamentul limfomului Hodgkin
Slovak	Resminostat	Liečba lymfómu Hodgkinovho typu
Slovenian	Resminostat	Zdravljenje Hodgkinovega limfoma
Spanish	Resminostat	Tratamiento del linfoma de Hodgkin
Swedish	Resminostat	Behandling av Hodgkin lymfom
Norwegian	Resminostat	Behandling av Hodgkin-lymfom
Icelandic	Resmínóstat	Meðferð við Hodgkins sjúkdómi

¹ At the time of designation