



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Resminostat for the treatment of hepatocellular carcinoma

On 27 September 2011, orphan designation (EU/3/11/913) was granted by the European Commission to 4SC AG, Germany, for resminostat for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?

Hepatocellular carcinoma is the most common type of primary cancer of the liver (a cancer that starts in the liver, rather than a cancer that has spread to the liver from another part of the body). It is more common in men than in women, and occurs mostly in people who have scarring of the liver (cirrhosis) or following infection with the hepatitis B or C viruses. Symptoms of the disease include pain and swelling in the abdomen, weight loss, weakness, loss of appetite and nausea (feeling sick).

Hepatocellular carcinoma is a severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, hepatocellular carcinoma affected approximately 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 51,000 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 in the EU for the treatment of hepatocellular carcinoma. The choice of treatment depended mainly on how advanced the disease was. The only therapies to cure the cancer were surgery to remove the tumour and liver transplantation, but these could only be carried out in very few patients. Other treatments included chemotherapy (medicines to treat cancer) and immunotherapy (medicines that stimulate the immune system to kill the cancer cells). Radiofrequency ablation (using a probe placed into the tumour to heat and destroy cancer cells) and ethanol injection were also used to remove small tumours.

*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).



The sponsor has provided sufficient information to show that resminostat might be of significant benefit for patients with hepatocellular carcinoma because it works in a different way to existing treatments and early studies suggest that it might be used in combination with specific existing treatments to improve the outcome of patients with this condition. These assumptions 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 hepatocellular carcinoma, 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 the medicine in patients with hepatocellular carcinoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of hepatocellular carcinoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 July 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

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Resminostat	Treatment of hepatocellular carcinoma
Bulgarian	РесминостаТ	Лечение на хепатоцелуларен карцином
Czech	Resminostat	Léčba hepatocelulárního karcinomu
Danish	Resminostat	Behandling af hepatocellulært karcinom
Dutch	Resminostat	Behandeling van hepatocellulair carcinoom
Estonian	Resminostat	Hepatotsellulaarse kartsinoomi ravi
Finnish	Resminostaatti	Hepatosellulaarisen karsinooman hoito
French	Resminostat	Traitement du cancer hépatocellulaire
German	Resminostat	Behandlung des Leberzellkarzinoms
Greek	Ρεσμινοστάτη	Θεραπεία του ηπατοκυτταρικού καρκινώματος
Hungarian	Resminostat	Hepatocellularis carcinoma kezelése
Italian	Resminostat	Trattamento del carcinoma epato cellulare
Latvian	Resminostats	Hepatocelulāras karcinomas ārstēšana
Lithuanian	Resminostatas	Hepatoceliulinės karcinomos gydymas
Maltese	Resminostat	Kura tal-karċinoma epatoċellulari
Polish	Resminostat	Leczenie raka wątrobowokomórkowego
Portuguese	Resminostat	Tratamento do carcinoma hepatocelular
Romanian	Resminostat	Tratamentul carcinomului hepatocelular
Slovak	Resminostat	Liečba hepatocelulárneho karcinómu
Slovenian	Resminostat	Zdravljenje hepatocelularnega karcinoma
Spanish	Resminostat	Tratamiento del carcinoma hepatocelular
Swedish	Resminostat	Behandling av hepatocellulärt karcinom
Norwegian	Resminostat	Behandling av hepatocellulært karsinom
Icelandic	Resmínóstat	Meðferð við lifrarfrumukrabbameini

¹ At the time of designation