

5 March 2013
EMA/COMP/227845/2011 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Salirasib for the treatment of pancreatic cancer

On 21 June 2011, orphan designation (EU/3/11/871) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for salirasib for the treatment of pancreatic cancer.

The sponsorship was transferred to Kadmon International Ltd, United Kingdom, in November 2013.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a juice that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread locally or to other parts of the body.

Pancreatic cancer is a very 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, pancreatic cancer affected approximately 1 in 10,000 people in the European Union (EU). This was 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 treating pancreatic cancer. The choice of treatment depended on several factors, including how advanced the disease is. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that salirasib might be of significant benefit for patients with pancreatic cancer because this medicine works in a different way to existing treatments

*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. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

and might be used in combination with existing treatments to improve the outcome of patients with this condition. 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?

Salirasib is expected to work by blocking 'the Ras signalling pathway'. This is a mechanism within cells that helps them to grow and survive. However, in cancer cells it works abnormally, leading to the growth of the cancer. Salirasib is expected to attach to specific proteins on the membrane of cancer cells and thus detach proteins called Ras. By detaching Ras, the medicine is expected to block the signalling pathway, thereby slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of salirasib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with salirasib in patients with pancreatic cancer were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 March 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:

Kadmon International Ltd
4th Floor
Haines House
21 John Street
London WC1N 2BP
United Kingdom
Telephone: +44 20 7692 1917
Telefax: +44 20 7692 1918

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	Salirasib	Treatment of pancreatic cancer
Bulgarian	Салиразиб	Лечение на рак на панкреаса
Czech	Salirasib	Léčba karcinomu pankreatu
Danish	Salirasib	Behandling af pancreascancer
Dutch	Salirasib	Behandeling van pancreaskanker
Estonian	Salirasib	Pankreasevähki ravi
Finnish	Salirasibi	Haimasyövän hoito
French	Salirasib	Traitement du cancer pancréatique
German	Salirasib	Behandlung des Pankreaskarzinoms
Greek	Σαλिरασίμπη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Salirasib	Hasnyálmirigyrák kezelése
Italian	Salirasib	Trattamento del cancro pancreatico
Latvian	Salirasibs	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Salirazibas	Kasos vėžio gydymas
Maltese	Salirasib	Kura tal-kanċer tal-frixa
Polish	Salirasyb	Leczenie raka trzustki
Portuguese	Salirasib	Tratamento do carcinoma do pâncreas
Romanian	Salirasib	Tratamentul cancerului pancreatic
Slovak	Salirasib	Liečba rakoviny pankreasu
Slovenian	Salirasib	Zdravljenje raka trebušne slinavke
Spanish	Salirasib	Tratamiento del cáncer de páncreas
Swedish	Salirasib	Behandling av pankreascancer
Norwegian	Salirasib	Behandling av pankreascancer
Icelandic	Salirasib	Meðferð briskrabbameins

¹ At the time of designation