



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Relacorilant for the treatment of pancreatic cancer

On 21 August 2019, orphan designation EU/3/19/2191 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for relacorilant for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, an organ that lies behind the stomach. The pancreas has two functions: to produce a fluid that helps with the digestion of food, and to produce hormones such as insulin. Because symptoms in the early stages of pancreatic cancer are vague (such as itching, fever and feeling sick), the majority of patients are diagnosed when the cancer has spread nearby or to other parts of the body.

Pancreatic cancer is a severe and life-threatening disease that can shorten life.

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

At the time of designation, pancreatic cancer affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 67,000 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 far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pancreatic cancer because early studies indicate that patients with pancreatic cancer that has spread and has not benefited from standard therapies show some response to the

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 518,400,000 (Eurostat 2019).



medicine. 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?

Relacorilant is a 'glucocorticoid receptor antagonist'. This means that it attaches to the same receptors (targets) that the hormone cortisol attaches to inside cells. When cortisol attaches to these receptors in pancreatic cancer cells it helps them to grow and resist cancer medicines. Relacorilant reduces cortisol's ability to attach to the receptors, and this is expected to slow the growth of the cancer.

What is the stage of development of this medicine?

The effects of relacorilant have been evaluated in experimental models.

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

At the time of submission, relacorilant was not authorised anywhere in the EU for the treatment of pancreatic cancer. Orphan designation of relacorilant had been granted in the US for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 July 2019, 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:

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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

¹ At the time of designation