



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

6 May 2020
EMADOC-628903358-1828

Public summary of opinion on orphan designation

Motixafortide for the treatment of pancreatic cancer

On 9 January 2020, orphan designation EU/3/19/2243 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for motixafortide for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is a cancer of the pancreas, a small 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. Due to the absence of symptoms in the early stages of pancreatic cancer, most 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 expectancy.

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

At the time of designation, pancreatic cancer affected approximately 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 93,000 people¹, 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 advanced pancreatic cancer. In early studies in patients with advanced

¹ 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).



pancreatic cancer, the tumour reduced in size when the medicine was used in combination with another cancer medicine called a 'checkpoint inhibitor.'

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?

This medicine blocks CXC chemokine receptor 4 (CXCR-4), which plays a role in preventing cells of the immune system (the body's natural defences) from attacking cancer cells and in promoting the spread of the cancer. By blocking CXCR4, the medicine stops the cancer switching off the immune cells, thereby increasing the immune system's ability to kill the cancer cells, and may slow down the spread of the cancer. Motixafortide also mobilises cancer cells to make them more prominent targets of other cancer medicines such as pembrolizumab.

What is the stage of development of this medicine?

The effects of motixafortide have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 December 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 languages², Norwegian and Icelandic

Language	Active ingredient	Indication
English	Motixafortide	Treatment of pancreatic cancer
Bulgarian	Мотиксафортид	Лечение на рак на панкреаса
Croatian	Motiksafortid	Liječenje raka gušterače
Czech	Motixafortid	Léčba karcinomu pankreatu
Danish	Motixafortid	Behandling af pancreascancer
Dutch	Motixafortide	Behandeling van pancreaskanker
Estonian	Motiksafortiid	Pankreasevähi ravi
Finnish	Motiksafortidi	Haimasyövän hoito
French	Motixafortide	Traitement du cancer pancréatique
German	Motixafortide	Behandlung des Pankreaskarzinoms
Greek	Μοτιξαφορτιδη	Θεραπεία του καρκίνου του παγκρέατος
Hungarian	Motixafortid	Hasnyálmirigyrák kezelése
Italian	Motixafortide	Trattamento del cancro pancreatico
Latvian	Motiksafortīds	Aizkuņģa dziedzerā vēža ārstēšana
Lithuanian	Motiksafortidas	Kasos vėžio gydymas
Maltese	Motiksafortide	Kura tal-kanċer tal-frixa
Polish	Motyksafortyd	Leczenie raka trzustki
Portuguese	Motixafortide	Tratamento do carcinoma do pâncreas
Romanian	Motixafortid	Tratamentul cancerului pancreatic
Slovak	Motixafortid	Liečba rakoviny pankreasu
Slovenian	Motiksafortid	Zdravljenje raka trebušne slinavke
Spanish	Motixafortida	Tratamiento del cáncer de páncreas
Swedish	Motixafortide	Behandling av pancreascancer
Norwegian	Motiksafortid	Behandling av pancreascancer
Icelandic	Motixafortid	Meðferð briskrabbameins

² At the time of designation