



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK for the treatment of pancreatic cancer

On 15 May 2009, orphan designation (EU/3/09/634) was granted by the European Commission to MBiotec GmbH, Germany, for S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK 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: producing a juice that helps with the digestion of food, and producing hormones such as insulin. About 95% of pancreatic cancers affect the cells that make the pancreatic juice. These are called adenocarcinomas.

Pancreatic cancer is a very severe and life-threatening disease that leads to poor long-term survival.

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 is equivalent to a total of around 66,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, several medicines were authorised for pancreatic cancer in the EU. The choice of treatment for pancreatic cancer depends on several factors, including the stage of the disease. Treatments may include surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK might be of significant benefit for patients with pancreatic cancer because it may increase patient survival. 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?

S-[2,3-bispalmitoyloxy-(2R)-propyl]-cysteinyl-GNNDESNISFKEK is a peptide (a protein fragment) that is expected to work by attaching to and activating two receptors called 'Toll-like receptor 2' and 'Toll-like receptor 6'. These receptors are part of the immune system (the body's natural defences) and their activation leads to an immune response. The medicine is injected directly into the pancreas, from where it is expected to stimulate the release of substances of the immune system that attack the cancer cells, particularly when it is combined with other cytotoxic (cell-killing) medicines.

*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 504,800,000 (Eurostat 2009).

What is the stage of development of this medicine?

The effects of S-[2,3-bisphosphatidyl-(2*R*)-propyl]-cysteinyl-GNNDENISFKEK have been evaluated in experimental models.

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

At the time of submission, this medicine was not authorised anywhere in the EU for pancreatic cancer 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 2 April 2009 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 Community) 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.

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**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active ingredient	Indication
English	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Treatment of pancreatic cancer
Bulgarian	S-[2,3-бисфалмитоилокси-(2R)-пропил]-цистеинил-GNNDENISFKEK	Лечение на рак на панкреаса
Czech	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Léčba karcinomu pankreatu
Danish	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Behandling af pancreascancer
Dutch	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Behandeling van pancreaskanker
Estonian	S-[2,3-bisphosphatidüloxi-(2R)-propüül]-tsüsteinüül-GNNDENISFKEK	Pankreasevähki ravi
Finnish	S-[2,3-bisphosphatidiloksi-(2R)-propyyl]-kysteinyyli-GNNDENISFKEK	Haimasyövän hoito
French	S-[2,3-bisphosphatidyl-(2R)-propyl]-cystéinyl-GNNDENISFKEK	Traitement du cancer pancréatique
German	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Behandlung des Pankreaskarzinoms
Greek	S-[2,3-bis φαλμιτο υλοξυ-(2R)-προπυλ]-κυστεϊνυλ-GNNDENISFKEK	Θεραπεία καρκίνου του παγκρέατος
Hungarian	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Hasnyálmirigyrák kezelése
Italian	S-[2,3-bisphosphatidilossi-(2R)-propil]-cisteinil-GNNDENISFKEK	Trattamento del cancro pancreatico
Latvian	S-[2,3-bisphosphatidiloksi-(2R)-propil]-cisteinil-GNNDENISFKEK	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	S-[2,3-bisphosphatidiloksi-(2R)-propil]-cisteinil-GNNDENISFKEK	Kasos vėžio gydymas
Maltese	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Kura tal-kanċer tal-frixa
Polish	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Leczenie raka trzustki
Portuguese	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Tratamento do carcinoma do pâncreas
Romanian	S-[2,3-bisphosphatidiloxi-(2R)-propil]-cisteinil-GNNDENISFKEK	Tratamentul cancerului pancreatic
Slovak	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Liečba rakoviny pankreasu
Slovenian	S-[2,3-bisphosphatidiloksi-(2R)-propil]-cisteinil-GNNDENISFKEK	Zdravljenje raka trebušne slinavke
Spanish	S-[2,3-bisphosphatidiloxi-(2R)-propil]-cisteinil-GNNDENISFKEK	Tratamiento del cáncer de páncreas
Swedish	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Behandling av pancreascancer
Norwegian	S-[2,3-bisphosphatidyl-(2R)-propyl]-cysteinyl-GNNDENISFKEK	Behandling av pancreascancer
Icelandic	S-[2,3-bisphosphatíloxý-(2R)-propýl]-cysteínýl-GNNDENISFKEK	Meðferð bráskabbameins