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Public summary of opinion on orphan designation

6,8-bis(benzylthio)octanoic acid for treatment of pancreatic cancer

On 14 December 2018, orphan designation (EU/3/18/2105) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for 6,8-bis(benzylthio)octanoic acid (also known as CPI-613) for 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. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread nearby or to other parts of the body.

Pancreatic cancer is a very 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 1.6 in 10,000 in the European Union (EU). This was equivalent to a total of around 83,000 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 this medicine might be of significant benefit for patients with pancreatic cancer. Results of early studies in patients suggest that patients whose disease had spread may live longer when treated with the medicine in combination with

*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 517,400,000 (Eurostat 2018).

standard treatment. 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?

The medicine, which is taken up in large amounts by cancer cells, blocks two enzymes that are needed for mitochondria (the energy-producing components within cells) to work properly. As a result, the cells cannot produce the energy needed to survive and grow. This is expected to lead to the death of cancer cells in patients with pancreatic cancer and thereby slow down the growth of the cancer.

What is the stage of development of this medicine?

The effects of the medicine 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 pancreatic cancer were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 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 [the 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	6,8-bis(benzylthio)octanoic acid	Treatment of pancreatic cancer
Bulgarian	6,8-бис(бензилтио)октанова киселина	Лечение на рак на панкреаса
Croatian	6,8-bis(benziltio)oktanska kiselina	Liječenje raka gušterače
Czech	6,8-bis(benziltio)oktanová kyselina	Léčba karcinomu pankreatu
Danish	6,8-bis(benzyl thiol)octansyre	Behandling af pancreascancer
Dutch	6,8-bis(benzylthio)octaanzuur	Behandeling van pancreaskanker
Estonian	6,8-bis-(bensüültio)-oktaanhape	Pankreasevähki ravi
Finnish	6,8-bis(bentsyylitio)oktaanihappo	Haimasyövän hoito
French	Acide 6,8-bis(benzylthio)octanoïque	Traitement du cancer pancréatique
German	6,8-Bis(benzylthio)octansäure	Behandlung des Pankreaskarzinoms
Greek	6,8-δι(βενζυλοθειο)οκτανοϊκό οξύ	Θεραπεία καρκίνου του παγκρέατος
Hungarian	6,8-bisz-(benzil-tio)-oktánsav	Hasnyálmirigyrák kezelése
Italian	Acido 6,8-bis(benziltio)ottanoico	Trattamento del cancro pancreatico
Latvian	6,8-bis(benziltio)kaprīliskābe	Aizkuņģa dziedzerā vēža ārstēšana
Lithuanian	6,8-bis(benziltio)oktano rūgštis	Kasos vėžio gydymas
Maltese	6,8-bis(benzylthio)octanoic acid	Kura tal-kanċer tal-frixa
Polish	Kwas 6,8-bis(benzylotio) oktanowy	Leczenie raka trzustki
Portuguese	Ácido 6,8-bis(benziltio)octanoico	Tratamento do carcinoma do pâncreas
Romanian	Acid 6,8-bis(benziltio)octanoic	Tratamentul cancerului pancreatic
Slovak	Kyselina 6,8-bis(benzylthio)oktánová	Liečba rakoviny pankreasu
Slovenian	6,8-bis(benziltio)oktanojska kislina	Zdravljenje raka trebušne slinavke
Spanish	Ácido 6,8-bis(benciltio)octanoico	Tratamiento del cáncer de páncreas
Swedish	6,8-bis(bensyltio)oktansyra	Behandling av pankreascancer
Norwegian	6,8-bis(benzyltio)oktansyre	Behandling av pankreascancer
Icelandic	6,8-bis(bensýlpíó)oktansýra	Meðferð briskrabbameins

¹ At the time of designation