



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

5 August 2020
EMADOC-628903358-2107

Public summary of opinion on orphan designation

2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidene)methyl)furan-2-yl)benzoate for the treatment of pancreatic cancer

On 24 March 2020, orphan designation EU/3/20/2259 was granted by the European Commission to MWB Consulting S.A.R.L., France, for 2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidene)methyl)furan-2-yl)benzoate (also known as GB1275) 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 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 104,000 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.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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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. Laboratory studies have suggested that adding the medicine to two other medicines used to treat pancreatic cancer (gemcitabine and paclitaxel) could prolong lives compared with just gemcitabine and paclitaxel.

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 attaches to a protein called CD11b which is found on the surface of certain cells of the immune system (the body's natural defences) called myeloid cells. In pancreatic cancer, myeloid cells do not work properly and prevent the cancer from being attacked by the immune system, and can promote cancer growth. By attaching to CD11b, the medicine stops the myeloid cells from moving to the site where the cancer is located and weakens their ability to promote cancer growth, allowing the immune system to attack the cancer. These effects are expected to slow down 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 the treatment of pancreatic cancer. Orphan designation had been granted in the USA for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 February 2020, 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	2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidene)methyl)furan-2-yl)benzoate	Treatment of pancreatic cancer
Bulgarian	2-хидрокси-N,N,N-триметилетан-1-аминиум (Z)-4-(5-((3-бензил-4-оксо-2-тиоксотиазалидин-5-илиден)метил) фуран-2-ил)бензоат	Лечение на рак на панкреаса
Croatian	2-hidroksi-N,N,N-trimetiletan-1-amino (Z)-4-(5-((3-benzil-4-okso-2-tioksotiazolidin-5-iliden)metil) furan-2-il)benzoat	Liječenje raka gušterače
Czech	2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-yliden)methyl)furan-2-yl)benzoát	Léčba karcinomu pankreatu
Danish	2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-yliden)methyl)furan-2-yl)benzoat	Behandling af pancreascancer
Dutch	2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylideen)methyl)furan-2-yl)benzoate	Behandeling van pancreaskanker
Estonian	2-hüdrosü-N,N,N-trimetüületaan-1-amiin (Z)-4-(5-((3-bensüül-4-okso-2-tioksotiasolidiin-5-üülideen)metüül)furaan-2-üül)bensoaat	Pankreasevähi ravi
Finnish	2-hydroksi-N,N,N-trimetyylietaani-1-aminium (Z)-4-(5-((3-bentsyyli-4-okso-2-tioksotiatsolidin-5-yylideeni)metyyli)furan-2-yyli)bentsoatti	Haimasyövän hoito
French	2-hydroxy-N,N,N-triméthyléthan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidène)méthyl)furan-2-yl)benzoate	Traitement du cancer pancréatique
German	2-Hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-yliden)methyl)furan-2-yl)Benzoat	Behandlung des Pankreaskarzinoms
Greek	2-υδροξυ-N,N,N-τριμεθυλαιθαν-1-αμμώνιο (Z)-4-(5-((3-βενζυλ-4-οξο-2-θειοξοθειαζολιδιν-5-υλιδενο)μεθυλ)φουραν-2-υλ)βενζοϊκό	Θεραπεία καρκίνου του παγκρέατος
Hungarian	2-hidroxi-N,N,N-trimetil-etán-1-aminium (Z)-4-(5-((3-benzil-4-oxo-2-tioxotiazolidin-5-ilidén)metil)furán-2-il)benzoát	Hasnyálmirigyrák kezelése
Italian	2-idrossi-N, N, N-trimetiletan-1-ammino (Z)-4-(5-((3-benzil-4-osso-2-tiosotiazolidin-5-ilidene)metil)furan-2-il)benzoato	Trattamento del cancro del pancreas

¹ At the time of designation

Language	Active ingredient	Indication
Latvian	2-hidroksi-N,N,N-trimetiletān-1-amīnija (Z)-4-(5-((3-benzil-4-okso-2-tioksotiazolidīn-5-ilidēn)metil)furān-2-il)benzoāts	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	2-hidroksi-N,N,N-trimetiletan-1-aminio (Z)-4-(5-((3-benzil-4-okso-2-tioksotiazolidin-5-iliden)metil)furan-2-il)benzoatas	Kasos vėžio gydymas
Maltese	2-idrossi-N,N,N-trimetiletan-1-aminju (Z)-4-(5-((3-benzil-4-osso-2-tijossotijažolidin-5-iliden)metil)furan-2-il)benžoat	Kura tal-kanċer tal-frixa
Polish	2-hydroksy-N, N, N-trimetyloetan-1-amin (Z)-4-(5-((3-benzyl-4-okso-2-tioksotiazolidyn-5-ylideno)metylo)furan-2-yl)benzoesan	Leczenie raka trzustki
Portuguese	Benzoato de 2-hidroxi-N,N,N-trimetiletan-1-aminio (Z)-4-(5-((3-benzil-4-oxo-2-tioxotiazolidin-5-ilideno)metil)furan-2-il)	Tratamento do carcinoma do pâncreas
Romanian	Benzoat de 2-hidroxi-N,N,N-trimetiletan-1-aminio (Z)-4-(5-((3-benzil-4-oxo-2-tioxotiazolidin-5-ilideno)methyl)furan-2-il)	Tratamentul cancerului pancreatic
Slovak	2-hydroxy-N,N,N-trimetyletán-1-amínium (Z)-4-(5-((3-benzyl-4-oxo-2-tioxotiazolidín-5-ylidén)metyl)furán-2-yl)benzoát	Liečba rakoviny pankreasu
Slovenian	2-hidroksi-N,N,N-trimetiletan-1-aminij (Z)-4-(5-((3-benzil-4-okso-2-tioksotiazolidin-5-iliden)metil)furan-2-il)benzoat	Zdravljenje raka trebušne slinavke
Spanish	2-hidroxi-N,N,N-trimetiletan-1-aminio (Z)-4-(5-((3-bencil-4-oxo-2-tioxotiazolidin-5-ilideno)metil)furan-2-il)benzoato	Tratamiento del cáncer de páncreas
Swedish	2-hydroxi-N,N,N-trimetyletan-1-aminium (Z)-4-(5-((3-bensyl-4-oxo-2-tioxotiazolidin-5-yliden)metyl)furan-2-yl)bensoat	Behandling av pankreascancer
Norwegian	2-hydroksy-N,N,N-trimetyletan-1-aminium (Z)-4-(5-((3-benzyl-4-okso-2-tioksotiazolidin-5-yliden) metyl)furan-2-yl)benzoat	Behandling av pankreaskreft
Icelandic	2-hýdroxý-N,N,N-trímetyletan-1-amíníum (Z)-4-(5-((3-bensýl-4-oxó-2-tíoxótíazólidín-5-ýliden)metýl)fúran-2-ýl)bensóat	Meðferð briskrabbameins