



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 October 2017
EMA/480448/2017

Public summary of opinion on orphan designation

Sodium 2-hydroxylinoleate for the treatment of pancreatic cancer

On 23 August 2017, orphan designation (EU/3/17/1911) was granted by the European Commission to Ability Pharmaceuticals SL, Spain, for sodium 2-hydroxylinoleate (also known as ABTL0812) for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is 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 very severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, pancreatic cancer affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 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 and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that sodium 2-hydroxylinoleate might be of significant benefit for patients with pancreatic cancer because laboratory studies show that it may reduce tumour growth when used in combination with currently authorised cancer 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 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).



(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?

This medicine is expected to work by reducing the activity of several proteins in the 'Akt/mTOR signalling pathway'. This is a mechanism within cells which is important in regulating their growth and survival. In many cancers, including pancreatic cancer, this pathway is overactive, allowing the cancer cells to grow uncontrollably. By reducing the activity of this pathway, the medicine is expected to slow down the progression of the cancer.

What is the stage of development of this medicine?

The effects of sodium 2-hydroxylinoleate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with sodium 2-hydroxylinoleate in patients with pancreatic cancer had been started.

At the time of submission, sodium 2-hydroxylinoleate was not authorised anywhere in the EU for pancreatic cancer. Orphan designation had been granted in the United States for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2017 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, on the medicine's [rare disease designations page](#).

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	Sodium 2-hydroxylinoleate	Treatment of pancreatic cancer
Bulgarian	Натрий 2-хидроксилинолеат	Лечение на рак на панкреаса
Croatian	Natrijev 2-hidroksilinoleat	Liječenje raka gušterače
Czech	2-hydroxilinoleát sodný	Léčba karcinomu pankreatu
Danish	Natrium 2-hydroxylinolat	Behandling af pancreascancer
Dutch	2-hydroxilinoleaat natrium	Behandeling van pancreaskanker
Estonian	Naatrium 2-hüdroksülinolaat	Pankreasevähi ravi
Finnish	Natrium 2-hydroksilinoლაatti	Haimasyövän hoito
French	2-hydroxylinoléate de sodium	Traitement du cancer pancréatique
German	Natrium 2-hydroxilinoლაt	Behandlung des Pankreaskarzinoms
Greek	2-υδροξυλινελαϊκό νάτριο	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Nátrium 2-hidroxilinoლაt	Hasnyálmirigyrák kezelése
Italian	2-idrossilinoლაeato di sodio	Trattamento del cancro pancreatico
Latvian	Nātrija 2-hidroksilinoლაēts	Aizkuģģa dziedzera vēģģa ārstēģģana
Lithuanian	Natrio 2-hidroksilinoლაeatas	Kasos vēģģio gydymas
Maltese	Sodium 2-hydroxylinoleate	Kura tal-kanċer tal-frixa
Polish	2-hydroksylinoლაeian sodu	Leczenie raka trzustki
Portuguese	2-hidroxilinoლაeato de sódio	Tratamento do carcinoma do pâncreas
Romanian	2-hidroxilinoლაeate de sodiu	Tratamentul cancerului pancreatic
Slovak	2-hydroxilinoლაeát sodný	Liečba rakoviny pankreasu
Slovenian	Natrijev 2-hidroksilinoლაt	Zdravljenje raka trebušne slinavke
Spanish	2-hidroxiinoლაeato sódico	Tratamiento del cáncer de pâncreas
Swedish	Natrium 2-hydroxilinoლაt	Behandling av pancreascancer
Norwegian	Natrium-2-hydroksylinoლაelat	Behandling av pancreascancer
Icelandic	Natríum 2-hýdroxýlínólati	Meðferð briskrabbameins

¹ At the time of designation