



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Fosgemcitabine palabenamide for the treatment of biliary tract cancer

On 24 March 2020, orphan designation EU/3/20/2262 was granted by the European Commission to Pharma Gateway AB, Sweden, for fosgemcitabine palabenamide (also known as NUC-1031) for the treatment of biliary tract cancer.

What is biliary tract cancer?

Biliary tract cancer affects the bile ducts and gallbladder. These are parts of the digestive system that transport and store bile, a fluid produced by the liver and released into the intestines to help digest fats. The cancer is characterised by features such as abnormal liver function tests, abdominal (belly) pain, yellowish discoloration of the skin and weight loss.

Biliary tract cancer is a long-term debilitating and life-threatening disease due to liver failure and problems caused when the cancer blocks the bile ducts.

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

At the time of designation, biliary tract cancer affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 78,000 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, no satisfactory methods were authorised in the EU for the treatment of biliary tract cancer. Some patients with early disease could undergo surgery to remove the cancer. Other treatments included chemotherapy medicines (medicines to treat cancer), although these were not authorised for biliary tract cancer.

*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).



How is this medicine expected to work?

Fosgemcitabine palabenamide is related to another cancer medicine, gemcitabine, that has been widely used in the EU. Like gemcitabine, it is taken up into dividing cells such as cancer cells and incorporated into their genetic material (DNA), interfering with their ability to grow and function normally. This results in death of the affected cells. Because of the differences in its structure, fosgemcitabine palabenamide is able to enter and kill some cancer cells that are resistant to the action of gemcitabine.

What is the stage of development of this medicine?

The effects of fosgemcitabine palabenamide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with fosgemcitabine palabenamide in patients with biliary tract cancer were ongoing.

At the time of submission, fosgemcitabine palabenamide was not authorised anywhere in the EU for the treatment of biliary tract cancer. Orphan designation of fosgemcitabine palabenamide had been granted in the United States for the treatment of biliary tract cancer.

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	Fosgemcitabine palabenamide	Treatment of biliary tract cancer
Bulgarian	Фосгемцитабин палабенамид	Лечение на рак на жлъчните пътища
Croatian	Fosgemcitabin palabenamid	Liječenje raka bilijarnog trakta
Czech	Fosgemcitabin palabenamid	Léčba karcinomu žlučových cest
Danish	Fosgemcitabinpalabenamid	Behandling af galdegangscancer
Dutch	Fosgemcitabine-palabenamide	Behandeling van galweg kanker
Estonian	Fosgemtsitabiinpalabenamiid	Sapiteede kasvaja ravi
Finnish	Fosgemsitabiini palabenamidi	Sappiteiden syövän hoito
French	Fosgemcitabine palabénamide	Traitement du cancer des voies biliaires
German	Fosgemcitabin-palabenamid	Behandlung von Tumoren der Gallenwege
Greek	Φωσγεμισταβίνη παλαβенаμίδη	Θεραπεία του καρκίνου της χοληφόρου οδού
Hungarian	Fosgemcitabin palabenamid	Epeúti rák kezelése
Italian	Fosgemcitabina palabenamide	Trattamento del carcinoma delle vie biliari
Latvian	Fosgemcitabīna palabenamīds	Žultsvadu sistēmas vēža ārstēšana
Lithuanian	Fosgemcitabinas palabenamidas	Tulžies latakų vėžio gydymas
Maltese	Fosgemcitabina palabenamur	Kura tal-kanċer tal-apparat tal-bili
Polish	Fosgemcytabina palabenamid	Leczenie raka dróg żółciowych
Portuguese	Fosgemcitabina palabenamida	Tratamento da neoplasia das vias biliares
Romanian	Fosgemcitabină palabenamidă	Tratamentul cancerului de căi biliare
Slovak	Fosgemcitabín palabenamid	Liečba karcinómu žlčových ciest
Slovenian	Fosgemcitabin palabenamid	Zdravljenje raka žolčnih vodov
Spanish	Fosgemcitabina palabenamida	Tratamiento del cáncer del árbol biliar
Swedish	Fosgemcitabin palabenamid	Behandling av gallvägscancer
Norwegian	Fosgemcitabinpalabenamid	Behandling av gallegangskreft
Icelandic	Fosgemsítabín palabenamíð	Meðferð við krabbameini í gallvegum

¹ At the time of designation