



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fimaporfin for the treatment of cholangiocarcinoma

On 29 August 2016, orphan designation (EU/3/16/1720) was granted by the European Commission to PCI Biotech AS, Norway, for fimaporfin for the treatment of cholangiocarcinoma.

What is cholangiocarcinoma?

Cholangiocarcinoma is a type of cancer that begins in the bile ducts. These are small tubes through which bile, which is produced by the liver and stored in the gall bladder, enters the intestine where it helps to digest fats. Symptoms usually only occur once the cancer is in its advanced stages, when it may spread to other parts of the body.

Cholangiocarcinoma is a life-threatening condition with a high mortality rate.

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

At the time of designation, cholangiocarcinoma affected less than 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 67,000 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 cholangiocarcinoma. Surgery was used to remove the cancer, and chemotherapy and radiotherapy were used to try to prevent the cancer from spreading to other parts of the body.

How is this medicine expected to work?

Fimaporfin is a photosensitising agent (a substance that changes when exposed to light). It is to be used with gemcitabine, a medicine already authorised for the treatment of several types of cancer.

*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 513,700,000 (Eurostat 2016).



When fimaporfin and gemcitabine are injected into a blood vessel, they are distributed throughout the body and reach the bile ducts. When a laser light is targeted at the bile duct cancer, fimaporfin is activated and generates reactive oxygen molecules that modify internal structures of the cancerous bile duct cells. This allows the accompanying gemcitabine to disrupt production of DNA in these cells, thus preventing them from multiplying and slowing 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 cholangiocarcinoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for cholangiocarcinoma 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 13 July 2016 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	Fimaporfin	Treatment of cholangiocarcinoma
Bulgarian	Фимапорфин	Лечение на холангиокарцином
Croatian	Fimaporfin	Liječenje kolangiokarcinoma
Czech	Fimaporfin	Léčba cholangiokarcinomu
Danish	Fimaporfin	Behandling af cholangiocarcinom
Dutch	Fimaporfine	Behandeling van cholangiocarcinoma
Estonian	Fimaporfiin	Kolangiokartsinoomi ravi
Finnish	Fimaporfiini	Kolangiokarsinooman hoito
French	Fimaporfine	Traitement du cholangiocarcinome
German	Fimaporfin	Behandlung von Gallengangskarzinom
Greek	Φιμαπορφίνη	Αγωγή για χολαγγειοκαρκίνωμα
Hungarian	Fimaporfin	Cholangiocarcinoma kezelése
Italian	Fimaporfina	Trattamento del colangiocarcinoma
Latvian	Fimaporfins	Žultsvada karcinomas ārstēšana
Lithuanian	Fimaporfinas	Cholangiokarcinomos gydymas
Maltese	Fimaporfin	Kura tal-kolanġokarċinoma
Polish	Fimaporfina	Leczenie raka dróg żółciowych
Portuguese	Fimaporfina	Tratamento do colangiocarcinoma
Romanian	Fimaporfină	Tratamentul colangiocarcinomului
Slovak	Fimaporfin	Liečba cholangiokarcinómu
Slovenian	Fimaporfin	Zdravljenje holangiokarcinoma
Spanish	Fimaporfina	Tratamiento del colangiocarcinoma
Swedish	Fimaporfin	Behandling av kolangiokarcinom
Norwegian	Fimaporfin	Behandling av kolangiokarsinom
Icelandic	Fímaporfin	Meðferð við gallrásakrabbameini

¹ At the time of designation