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Public summary of opinion on orphan designation

Pemigatinib for the treatment of biliary tract cancer

On 24 August 2018, orphan designation (EU/3/18/2066) was granted by the European Commission to Incyte Biosciences Distribution B.V., the Netherlands, for pemigatinib for the treatment of biliary tract cancer.

What is biliary tract cancer?

Biliary tract cancer is cancer of 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 after a meal to help digest fats. The cancer is characterised by various features such as abnormal liver function tests, pain in the belly, 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.

*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).

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How is this medicine expected to work?

Pemigatinib belongs to a group of medicines called protein kinase inhibitors. It works by blocking enzymes known as protein kinases, particularly those that are part of receptors (targets) called fibroblast growth factor receptors (FGFRs). FGFRs are found on the surface of cancer cells and are involved in the growth and spread of the cancer cells. By blocking the tyrosine kinases in FGFRs, pemigatinib is expected to reduce the growth and spread 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 pemigatinib including patients with biliary tract cancer were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2019 recommending the granting of this designation.

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pemigatinib	Treatment of biliary tract cancer
Bulgarian	Пемигатиниб	Лечение на рак на жлъчните пътища
Croatian	Pemigatinib	Liječenje raka bilijarnog trakta
Czech	Pemigatinib	Léčba karcinomu žlučových cest
Danish	Pemigatinib	Behandling af galdegangscancer
Dutch	Pemigatinib	Behandeling van galweg kanker
Estonian	Pemigatiniib	Sapiteede kasvaja ravi
Finnish	Pemigatinibi	Sappiteiden syövän hoito
French	Pemigatinib	Traitement du cancer des voies biliaires
German	Pemigatinib	Behandlung von Tumoren der Gallenwege
Greek	Πεμιγκατινίμπη	Θεραπεία του καρκίνου της χοληφόρου οδού
Hungarian	Pemigatinib	Epeúti rák kezelése
Italian	Pemigatinib	Trattamento del carcinoma delle viebiliari
Latvian	Pemigatinibs	Žultsvadu sistēmas vēža ārstēšana
Lithuanian	Pemigatinibas	Tulžies latakų vėžio gydymas
Maltese	Pemigatinib	Kura tal-kanċer tal-apparat tal-bili
Polish	Pemigatynib	Leczenie raka dróg żółciowych
Portuguese	Pemigatinib	Tratamento da neoplasia das vias biliares
Romanian	Pemigatinib	Tratamentul cancerului de căi biliare
Slovak	Pemigatinib	Liečba karcinómu žlčových ciest
Slovenian	Pemigatinib	Zdravljenje raka žolčnih vodov
Spanish	Pemigatinib	Tratamiento del cáncer del árbol biliar
Swedish	Pemigatinib	Behandling av gallvägscancer
Norwegian	Pemigatinib	Behandling av gallegangskreft
Icelandic	Pemigatíníb	Meðferð við krabbameini í gallvegum

[1] At the time of designation