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Public summary of opinion on orphan designation

Infigratinib for the treatment of cholangiocarcinoma

On 21 August 2020, orphan designation EU/3/20/2329 was granted by the European Commission to YES Pharmaceutical Development Services GmbH, Germany, for infigratinib 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 have 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 approximately 1.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 88,000 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 the application for orphan designation, no satisfactory methods were authorised in the EU for the treatment of cholangiocarcinoma. Surgery was used to remove the cancer, and chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation) were used to try to prevent the cancer from spreading to other parts of the body.

*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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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How is this medicine expected to work?

Some patients with cholangiocarcinoma have mutations (changes) in the genes responsible for the production of proteins called fibroblast growth factor receptors (FGFRs). These mutations are thought to play a role in the development of cancer, including cholangiocarcinoma.

This medicine blocks the action of FGFR 1, 2, and 3. By blocking these FGFRs, the medicine is expected to prevent cancer cells from multiplying and the development of blood vessels that help tumours grow. This is expected to slow down the worsening of the disease.

What is the stage of development of this medicine?

The effects of infigratinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with infigratinib in patients with cholangiocarcinoma were ongoing.

At the time of submission, infigratinib was not authorised anywhere in the EU for the treatment of cholangiocarcinoma. Orphan designation of infigratinib had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 16 July 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

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Infigratinib	Treatment of cholangiocarcinoma
Bulgarian	Инфигратиниб	Лечение на холангиокарцином
Croatian	Infigratinib	Liječenje kolangiokarcinoma
Czech	Infigratinib	Léčba cholangiokarcinomu
Danish	Infigratinib	Behandling af cholangiocarcinom
Dutch	Infigratinib	Behandeling van cholangiocarcinoma
Estonian	Infigratiniib	Kolangiokartsinoomi ravi
Finnish	Infigratinibi	Kolangiokarsinooman hoito
French	Infigratinib	Traitement du cholangiocarcinome
German	Infigratinib	Behandlung von Gallengangskarzinom
Greek	Ινφιγκρατινίμπη	Θεραπεία του χολαγγειοκαρκινώματος
Hungarian	Infigratinib	Cholangiocarcinoma kezelése
Italian	Infigratinib	Trattamento del colangiocarcinoma
Latvian	Infigratinibs	Žultsvada karcinomas ārstēšana
Lithuanian	Infigratinibas	Cholangiokarcinomos gydymas
Maltese	Infigratinib	Kura tal-kolanġokarċinoma
Polish	Infrigatinib	Leczenie raka dróg żółciowych
Portuguese	Infigratinib	Tratamento do colangiocarcinoma
Romanian	Infigratinib	Tratamentul colangiocarcinomului
Slovak	Infigratinib	Liečba cholangiokarcinómu
Slovenian	infigratinib	Zdravljenje holangiokarcinoma
Spanish	Infigratinib	Tratamiento del colangiocarcinoma
Swedish	Infigratinib	Behandling av kolangiokarcinom
Norwegian	Infigratinib	Behandling av kolangiokarsinom
Icelandic	Infigratinib	Meðferð við gallrásakrabbameini

[1] At the time of designation