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# Public summary of opinion on orphan designation

## Ivosidenib for the treatment of biliary tract cancer

On 21 March 2018, orphan designation (EU/3/18/1994) was granted by the European Commission to QRC Consultants Ltd, United Kingdom, for ivosidenib 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 which is 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 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 biliary tract cancer. Some patients with early disease could undergo surgery to remove the cancer. Other treatments included chemotherapy (medicines to treat cancer).

### How is this medicine expected to work?

Some patients with biliary tract cancer have a fault in a gene called *IDH1*, which causes production of an abnormal IDH1 protein. The abnormal protein makes a substance, 2-hydroxyglutarate, that causes

*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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cells to become cancerous. Ivosidenib is expected to block the activity of the abnormal IDH1 protein and so reduces production of 2-hydroxyglutarate, thereby preventing formation of cancer cells.

## What is the stage of development of this medicine?

The effects of ivosidenib have been evaluated in experimental models.

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

At the time of submission, ivosidenib was not authorised anywhere in the EU for biliary tract cancer 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 15 February 2018 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 | Ivosidenib | Treatment of biliary tract cancer |
| Bulgarian | Ивосидениб | Лечение на рак на жлъчните пътища |
| Croatian | Ivosidenib | Liječenje raka bilijarnog trakta |
| Czech | Ivosidenib | Léčba karcinomu žlučových cest |
| Danish | Ivosidenib | Behandling af galdegangscancer |
| Dutch | Ivosidenib | Behandeling van galweg kanker |
| Estonian | Ivosideniib | Sapiteede kasvaja ravi |
| Finnish | Ivosidenibi | Sappiteiden syövän hoito |
| French | Ivosidenib | Traitement du cancer des voies biliaires |
| German | Ivosidenib | Behandlung von Tumoren der Gallenwege |
| Greek | Ιβοσιδενίμπη | Θεραπεία του καρκίνου της χοληφόρου οδού |
| Hungarian | Ivosidenib | Epeúti rák kezelése |
| Italian | Ivosidenib | Trattamento del carcinoma delle viebiliari |
| Latvian | Ivosidenibs | Žultsvadu sistēmas vēža ārstēšana |
| Lithuanian | Ivosidenibas | Tulžies latakų vėžio gydymas |
| Maltese | Ivosidenib | Kura tal-kanċer tal-apparat tal-bili |
| Polish | Iwosidenib | Leczenie raka dróg żółciowych |
| Portuguese | Ivosidenib | Tratamento da neoplasia das vias biliares |
| Romanian | Ivosidenib | Tratamentul cancerului de căi biliare |
| Slovak | Ivosidenib | Liečba karcinómu žlčových ciest |
| Slovenian | Ivosidenib | Zdravljenje raka žolčnih vodov |
| Spanish | Ivosidenib | Tratamiento del cáncer del árbol biliar |
| Swedish | Ivosidenib | Behandling av gallvägscancer |
| Norwegian | Ivosidenib | Behandling av gallegangskreft |
| Icelandic | Ívósídeníb | Meðferð við krabbameini í gallvegum |

[1] At the time of designation