EMA/95344/2021
EMEA/H/C/005266

Pemazyre (*pemigatinib*)

An overview of Pemazyre and why it is authorised in the EU

What is Pemazyre and what is it used for?

Pemazyre is a cancer medicine used to treat adults with cholangiocarcinoma (biliary tract cancer or cancer of the bile ducts) when the cancer cells have an abnormal form of a receptor (target) called FGFR2 on their surface. Pemazyre is used when the cancer has spread to other parts of the body or cannot be removed by surgery and has worsened after previous treatment with at least one cancer medicine.

Cholangiocarcinoma is rare, and Pemazyre was designated an 'orphan medicine' (a medicine used in rare diseases) on 24 August 2018. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3182066.

Pemazyre contains the active substance pemigatinib.

How is Pemazyre used?

Pemazyre is available as tablets to be taken by mouth. The medicine can only be obtained with a prescription and treatment must be started by a doctor experienced in diagnosis and treatment of the disease. It is taken in three-week cycles consisting of two weeks where Pemazyre is taken daily followed by a week without the medicine. Treatment can continue for as long as the patient benefits from it and side effects are manageable.

For more information about using Pemazyre, see the package leaflet or contact your doctor or pharmacist.

How does Pemazyre work?

The active substance in Pemazyre, pemigatinib, belongs to a group of medicines called protein kinase inhibitors. It works by blocking the activity of receptors called fibroblast growth factor receptors (FGFRs). Abnormal FGFRs are found on the surface of cancer cells and are involved in the growth and spread of the cancer. By blocking their activity, Pemazyre reduces the growth and spread of the cancer.

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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

What benefits of Pemazyre have been shown in studies?

Pemazyre was effective in reducing the size of the cancer lesions in one main study involving 108 patients with biliary tract cancer with abnormal FGFR2. Around 37% of patients had a shrinkage of their cancer that lasted on average for 8 months.

What are the risks associated with Pemazyre?

The most common side effects with Pemazyre (which may affect more than 1 in 10 people) are high or low levels of phosphate in the blood, alopecia (hair loss), diarrhoea, problems with the nails, tiredness, nausea (feeling sick), dysgeusia (taste disturbances), stomatitis (inflammation of the lining of the mouth), constipation, joint pain, dry mouth, eyes and skin, rash and numbness on the palms and soles, low levels of sodium in the blood and high levels of creatinine in the blood, which could indicate kidney problems.

Pemazyre must not be taken with the herbal medicine St John's wort. For the full list of side effects and restrictions with Pemazyre, see the package leaflet.

Why is Pemazyre authorised in the EU?

Pemazyre is considered effective in patients with biliary tract cancer that progressed after at least one prior treatment and for whom there are no other authorised treatments. Patients can tolerate the side effects of the medicine when they are closely monitored and treated if needed. The European Medicines Agency therefore decided that Pemazyre's benefits are greater than its risks and it can be authorised for use in the EU.

Pemazyre has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Pemazyre?

Since Pemazyre has been given conditional authorisation, the company that markets the medicine will provide the final results of the main study on the safety and effectiveness of Pemazyre and the results of a study comparing Pemazyre with gemcitabine and cisplatin (other cancer medicines) in patients with newly diagnosed biliary tract cancer.

What measures are being taken to ensure the safe and effective use of Pemazyre?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Pemazyre have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Pemazyre are continuously monitored. Side effects reported with Pemazyre are carefully evaluated and any necessary action taken to protect patients.

Other information about Pemazyre

Pemazyre received a conditional marketing authorisation valid throughout the EU on 26 March 2021.

Further information on Pemazyre can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/pemazyre.

This overview was last updated in 03-2021.