25 February 2021
EMA/35501/2021 Rev. 1
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pemazyre

pemigatinib

On 28 January 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a conditional[2] marketing authorisation for the medicinal product Pemazyre[3], intended for the second-line treatment of advanced or metastatic cholangiocarcinoma characterized by fusion or rearrangements of fibroblast growth factor receptor 2. The applicant for this medicinal product is Incyte Biosciences Distribution B.V.

Pemazyre will be available as 4.5 mg, 9 mg and 13.5 mg tablets. The active substance of Pemazyre is pemigatinib, a protein kinase inhibitor (ATC code: L01EX20) which is a kinase inhibitor of FGFR 1, 2 and 3 that inhibits FGFR phosphorylation and signalling and decreases viability of cells expressing FGFR genetic alterations, including point mutations, amplifications, and fusions or rearrangements.

The benefits with Pemazyre are its ability to increase the number of patients with a complete or partial response after first-line treatment, which is maintained for a median of 8 months.

The most common side effects are hyperphosphatemia, alopecia, diarrhoea, nail toxicity, fatigue, nausea, dysgeusia, stomatitis, constipation, dry mouth, dry eye, arthralgia, hypophosphataemia, dry skin and palmar-plantar erythrodysaesthesia syndrome.

The full indication is:

> Pemazyre monotherapy is indicated for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy[4].

Pemazyre should be prescribed by physicians experienced in the diagnosis and treatment of patients with

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] A conditional marketing authorisation is granted to a medicinal product that fulfils an unmet medical need when the benefit to public health of immediate availability outweighs the risk inherent in the fact that additional data are still required. The marketing authorisation holder is likely to provide comprehensive clinical data at a later stage.
[3] This product was designated as orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained
[4] This document was updated to reflect changes to the text of the indication for Pemazyre adopted by CHMP on 25 February 2021

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biliary tract cancer.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.