



pemigatinib (Pemazyre™)

EOCCO POLICY



Policy Type: PA/SP

Pharmacy Coverage Policy: EOCCO191

Description

Pemigatinib (Pemazyre) is an orally administered fibroblast growth factor receptor 2 (FGFR2) inhibitor, with activity against FGFR2 fusions or rearrangements in cholangiocarcinoma cells.

Length of Authorization

- N/A

Quantity Limits

Product Name	Dosage Form	Indication	Quantity Limit
Pemigatinib (Pemazyre)	13.5 mg tablet	Previously treated, unresectable, locally advanced or metastatic cholangiocarcinoma in adults with FGFR2 fusions or rearrangements	14 tablets/21 days
	9 mg tablet		
	4.5 mg tablet		

Initial Evaluation

- I. Pemigatinib (Pemazyre) is considered investigational when used for all conditions, including but not limited to cholangiocarcinoma.

Renewal Evaluation

- I. N/A

Supporting Evidence

- I. Pemigatinib (Pemazyre) is the first targeted therapy for cholangiocarcinoma that harbors FGFR2 fusions or rearrangements. Pemigatinib (Pemazyre) is a second-line chemotherapy option. Guideline preferred first line chemotherapy is gemcitabine and cisplatin, while second-line options include mFOLFOX, FOLFIRI, and regorafenib (Stivarga).
- II. Pemigatinib (Pemazyre) was evaluated in FIGHT-202, an open-label, single-arm, multi-cohort Phase 2 trial. Patients (N=146) with locally advanced or metastatic CCA, previously treated with at least 1 chemotherapy were included. FDA approval was based on the overall response rate (ORR) in patients with FGFR2 gene fusion or rearrangements.

- III. The primary efficacy endpoint was objective response rate (ORR). Secondary endpoints were progression-free survival (PFS), overall survival (OS), and duration of response (DOR). Based on analysis of this clinical trial data, quality of the evidence is considered low given the lack of comparator and open-label trial design, as well as, the lack of clinically meaningful outcomes in morbidity, mortality, and quality of life – medication efficacy has not yet been confirmed.
- IV. Pemigatinib (Pemazyre) received accelerated approval from the FDA based on ORR and DOR. Continued approval for this drug may be contingent upon verification of clinical benefit in confirmatory trials. There is a Phase 3 trial underway to assess pemigatinib (Pemazyre) monotherapy versus gemcitabine + cisplatin in the first-line treatment of CCA with FGFR2 alterations.
- V. The safety profile of pemigatinib (Pemazyre) was based on adverse reactions observed in all cohorts during CT (N=146). The most common adverse events (≥20% incidence) included hyperphosphatemia, alopecia, nausea, diarrhea, nail toxicity, back pain, fatigue, dysgeusia, dry eyes, and serous retinal detachment. There are no specific contraindications to pemigatinib (Pemazyre); however, warnings and precautions include: ocular toxicity, hyperphosphatemia, GI toxicity and renal function. Pemigatinib (Pemazyre) showed 9% treatment discontinuation rate, 14% dose reductions rate, and 42% dose interruption rate due to adverse events.
- VI. As of June 2020, The National Comprehensive Cancer Network (NCCN) treatment guideline for hepatobiliary cancer has included pemigatinib (Pemazyre) as second-line treatment with a Category 2A recommendation. Pemigatinib (Pemazyre) is useful in treatment of tumor with confirmed FGFR2 fusions or rearrangements; and which are refractory to first line chemotherapy.

Investigational or Not Medically Necessary Uses

- I. Pemigatinib (Pemazyre) has not been sufficiently studied for safety and efficacy for any other condition to date.

References

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3. Razumilava, N; Gores, GJ. "[Cholangiocarcinoma](#)". Lancet. 2014, 383, (9935): 68–79.
4. [Patel N; Benipal B. 2019](#), Incidence of Cholangiocarcinoma in the USA from 2001 to 2015: A US Cancer Statistics Analysis of 50 States. Cureus. 2019;11(1): e3962.
5. Valle J, Wasan H, et al. Cisplatin plus gemcitabine vs gemcitabine for biliary tract cancer. N. Engl. J. Med. 2010; 362(14): 1273-81.
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7. Nakamura H, Arai Y, Totoki Y, et al. Genomic spectra of biliary tract cancer. Nature Genetics 2015; 47: 1003-10.
8. Krook MA, Lenyo A, Wilberding M, et al. Efficacy of FGFR inhibitors and combination therapies for acquired resistance in FGFR2-fusion cholangiocarcinoma. Molecular Cancer Therapeutics 2020; 19: 847-57.



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- 9. NCCN guidelines for hepatobiliary cancers, version 2.2020.
www.nccn.org/professionals/physician_gls/pdf/hepatobiliary.pdf
- 10. Abou-alfa GK et al. Pemigatinib for previously treated, locally advanced or metastatic cholangiocarcinoma: a multicenter, open-label, phase 2 study. *Lancet Oncol.* 2020 May;21(5):671-684. (NCT02924376)
- 11. 11. A study to evaluate the efficacy and safety of pemigatinib versus chemotherapy in unresectable or metastatic cholangiocarcinoma (FIGHT-302), clinicaltrials.gov; NCT03656536. <https://clinicaltrials.gov/ct2/show/NCT03556536>.

Policy Implementation/Update:

Action and Summary of Changes	Date
Policy created	06/2020