

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Futibatinib for previously treated advanced cholangiocarcinoma with FGFR2 fusion or rearrangement [ID6302]

Final scope

Remit/evaluation objective

To appraise the clinical and cost effectiveness of futibatinib within its marketing authorisation for treating cholangiocarcinoma.

Background

Cholangiocarcinoma is cancer of the bile duct. It mainly affects people aged over 65. Most people already have advanced cholangiocarcinoma when they are diagnosed because early disease is often asymptomatic. When symptoms occur, they include jaundice, itchy skin, weight loss, abdominal pain, fatigue and fever.

Cholangiocarcinoma can be classified into 3 subtypes, depending on which part of the bile duct the cancer starts in. Intrahepatic cholangiocarcinoma (iCCA) starts in the bile ducts inside the liver, peri-hilar cholangiocarcinoma starts just outside the liver (where the left and right hepatic ducts meet) and distal cholangiocarcinoma starts in the bile ducts near the bowel.^{1,2} The overall incidence of cholangiocarcinoma is increasing with currently around 2,800 people diagnosed each year in England.³ There is a wide range of estimates for the proportion of cases which are iCCA, ranging from 10%-74%.^{4,5} Of these, ~10-15% will have fusion or rearrangements of fibroblast growth factor receptors (FGFRs).⁶ This is less common in other types of cholangiocarcinoma. FGFRs play a role in the growth and spread of the cancer cells. Of people diagnosed in England in 2012, 28.5% of men and 24.6% of women survived for 1 year or more. Of people diagnosed in England in 2008, 6.6% of men and 4.4% of women survived for 5 years or more.⁷

Surgery is currently the only curative treatment for cholangiocarcinoma.⁸ When surgery is not an option people can be offered systemic chemotherapy (typically gemcitabine and cisplatin). After systemic chemotherapy, British and European guidelines recommend that FGFR inhibitors, such as pemigatinib, are considered for people with FGFR2 alterations.^{5,9} NICE technology appraisal 722 recommends pemigatinib as an option for treating advanced cholangiocarcinoma with FGFR2 fusion or rearrangement after systemic therapy in adults. Alternatively, people may be offered modified folinic acid, fluorouracil and oxaliplatin (mFOLFOX) in the second line setting.

The technology

Futibatinib (Lytgobi, Taiho Pharma Europe, Limited) is indicated for the treatment of adult patients with locally advanced or metastatic cholangiocarcinoma with FGFR2 fusion or rearrangement that has progressed after at least 1 prior systemic therapy.

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Intervention(s)	Futibatinib
Population(s)	Adults with locally advanced or metastatic cholangiocarcinoma with FGFR2 fusion or rearrangement that has progressed after at least 1 prior systemic therapy
Comparators	<ul style="list-style-type: none"> • Pemigatinib • Modified FOLFOX regimen (folinic acid, fluorouracil and oxaliplatin) • Best supportive care
Outcomes	<p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • overall survival • progression-free survival • response rates • adverse effects of treatment • health-related quality of life.
Economic analysis	<p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>If the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost comparison may be carried out.</p> <p>The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p> <p>The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.</p> <p>The availability and cost of biosimilar and generic products should be taken into account.</p>

Other considerations	<p>Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.</p>
Related NICE recommendations	<p>Related technology appraisals:</p> <p>Pemigatinib for treating relapsed or refractory advanced cholangiocarcinoma with FGFR2 fusion or rearrangement (2021) NICE technology appraisal guidance 722</p> <p>Related technology appraisals in development:</p> <p>Ivosidenib for treating advanced cholangiocarcinoma with an IDH1 mutation after at least 1 therapy NICE technology appraisal guidance ID6164. Publication expected December 2023.</p> <p>Durvalumab with gemcitabine and cisplatin for treating unresectable or advanced biliary tract cancer NICE technology appraisal guidance ID4031. Publication tbc.</p> <p>Pembrolizumab with gemcitabine and cisplatin for untreated advanced biliary tract cancer. NICE technology appraisal guidance ID4034. Publication tbc.</p> <p>Related Interventional Procedures:</p> <p>Selective internal radiation therapy for unresectable primary intrahepatic cholangiocarcinoma (2018) Interventional procedures guidance IPG630</p> <p>Photodynamic therapy for bile duct cancer (2005) Interventional procedures guidance IPG134</p> <p>Endoscopic bipolar radiofrequency ablation for treating biliary obstruction caused by cholangiocarcinoma or pancreatic adenocarcinoma Interventional procedures guidance in development.</p>
Related National Policy	<p>The NHS Long Term Plan, 2019. NHS Long Term Plan</p> <p>NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019)</p>

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References

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- 2 Cancer Research UK (2023) Types of bile duct cancer. <https://www.cancerresearchuk.org/about-cancer/bile-duct-cancer/types> [Accessed September 2023]
- 3 Cancer Research UK (2023) What is bile duct cancer? <https://www.cancerresearchuk.org/about-cancer/bile-duct-cancer/about> [Accessed September 2023]
- 4 Razumilava, N. and Gores, G.J., 2013. Classification, diagnosis, and management of cholangiocarcinoma. *Clinical gastroenterology and hepatology*, 11(1), pp.13-21.8.
- 5 Rushbrook, S. M., et al. (2023). British Society of Gastroenterology guidelines for the diagnosis and management of cholangiocarcinoma. *Gut*.
- 6 Goyal, L., Kongpetch, S., Crolley, V.E. and Bridgewater, J., 2021. Targeting FGFR inhibition in cholangiocarcinoma. *Cancer treatment reviews*, 95, p.102170. <https://doi.org/10.1016/j.ctrv.2021.102170>
- 7 Public Health England Age-standardised incidence rates, one- and five-year survival, all patients diagnosed with upper gastrointestinal cancers, England <http://www.ncin.org.uk/view?rid=3022> [accessed September 2023]
- 8 BMJ Best Practice: Cholangiocarcinoma <https://bestpractice.bmj.com/topics/en-gb/721> [Accessed September 2023]
- 9 Vogel, A., et al. 2023. Biliary tract cancer: ESMO Clinical Practice Guideline for diagnosis, treatment and follow-up. *Annals of Oncology*, 34(2), pp.127-140. <https://doi.org/10.1016/j.annonc.2022.10.506>