NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Futibatinib for previously treated advanced cholangiocarcinoma with FGFR2 fusion or rearrangement

Draft scope

Draft 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] Approximately 10% of these cases are iCCA, and ~10-15% of those will have fusion or rearrangements of fibroblast growth factor receptors (FGFRs).[4] 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.[5]

Surgery is currently the only curative treatment for cholangiocarcinoma.[6] When surgery is not an option people can be offered systemic chemotherapy (typically gemcitabine and cisplatin). After systemic chemotherapy, ESMO Clinical Practice Guidelines state that FGFR inhibitors are recommended for the treatment of patients with FGFR2 fusions, where available.[7] NICE technology appraisal 722 recommends pemigatinib 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 Oncology Europe) is indicated for the treatment of locally advanced or metastatic cholangiocarcinoma with FGFR2 fusion or rearrangement that has progressed after at least 1 prior systemic therapy.

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	• Pemigatinib • Modified FOLFOX regimen (folinic acid, fluorouracil and oxaliplatin) • Best supportive care
Outcomes	The outcome measures to be considered include: • overall survival • progression-free survival • response rates • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. 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. 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. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account. The availability and cost of biosimilar and generic products should be taken into account.

Other considerations	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.
Related NICE recommendations	**Related technology appraisals:** Pemigatinib for treating relapsed or refractory advanced cholangiocarcinoma with FGFR2 fusion or rearrangement (2021) NICE technology appraisal guidance 722 **Related technology appraisals in development:** Ivosidenib for treating advanced cholangiocarcinoma with an IDH1 mutation after at least 1 therapy NICE technology appraisal guidance ID6164. Publication expected December 2023. **Related Interventional Procedures:** Selective internal radiation therapy for unresectable primary intrahepatic cholangiocarcinoma (2018) Interventional procedures guidance IPG630 Photodynamic therapy for bile duct cancer (2005) Interventional procedures guidance IPG134 Endoscopic bipolar radiofrequency ablation for treating biliary obstruction caused by cholangiocarcinoma or pancreatic adenocarcinoma Interventional procedures guidance in development.
Related National Policy	The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019)

Questions for consultation

Where do you consider futibatinib will fit into the existing care pathway for advanced cholangiocarcinoma?

Which treatments are considered to be established clinical practice in the NHS for locally advanced or metastatic cholangiocarcinoma with FGFR2 fusion or rearrangement that has progressed after at least 1 prior systemic therapy?

Should best supportive care be included as a comparator? If so, what does best supportive care consist of?

Is mFOLFOX a relevant comparator? Would any other chemotherapy regimens be used?

Is genetic testing for FGFR2 fusion or rearrangement routinely done in NHS clinical practice?

Would futibatinib be a candidate for managed access?

Do you consider that the use of futibatinib can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which futibatinib will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE is considering evaluating this technology through its cost comparison evaluation process.

Please provide comments on the appropriateness of appraising this topic through this process.

(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of the comparators? Or in what way is it different to the comparators?
- Will the intervention be used in the same place in the treatment pathway as the comparator(s)? Have there been any major changes to the treatment pathway recently? If so, please describe.
- Will the intervention be used to treat the same population as the comparator(s)?
- Overall is the technology likely to offer similar or improved health benefits compared with the comparators?
- Would it be appropriate to use the cost-comparison methodology for this topic?

References

1 Khan, SA, Tavolari, S, Brandi, G. Cholangiocarcinoma: Epidemiology and risk factors. Liver Int. 2019; 39(Suppl. 1): 19– 31. https://doi.org/10.1111/liv.14095

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 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

5 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]

6 BMJ Best Practice: Cholangiocarcinoma https://bestpractice.bmj.com/topics/en-gb/721 [Accessed September 2023]

7 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

