NICE National Institute for Health and Care Excellence

Highly Specialised Technologies (HST) criteria checklist
Ivosidenib for cholangiocarcinoma [ID6164]

Introduction

The NICE HST criteria checklist is to highlight where a technology meets/partially meets or does not meet the criteria for routing to the HST programme. Its purpose is to show the details of why a technology may not be appropriate for HST evaluation, but also where it has been identified as suitable. For more information, please see section 7 of NICE health technology evaluation topic selection: the manual

Key – Please use the colour key to advise if the technology meets the criteria

Met	There is clear and strong evidence that the criterion is met
Not met	There is some, but not enough clear evidence that the criterion is met or There is no evidence or limited evidence that the criterion is met.

MA wording: MA not yet granted

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
1.	The disease is very rare defined by 1:50,000 in England	• Cholangiocarcinoma is a subset of bile duct cancer and is the disease considered here as oppose to the indication specific population which is considered in the next criterion. • NHS Digital cancer registration statistics recorded 2618 cases of intrahepatic and extrahepatic bile duct carcinoma (ICD10 C22.1 and C24.0) in 2020.[1]	Not met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
		• 1945 of these cases were intrahepatic bile duct carcinoma (ICD10 C22.1) and 672 were extrahepatic bile duct carcinoma (ICD10 C24.0) • This criterion considers the condition as a whole, not the indication specific population • As the number of cholangiocarcinoma cases diagnosed each year exceeds 1,120 (the number which would represent a 1:50,000 prevalence in England) this criterion is not met. 1. NHS Digital – Cancer registration statistics, England 2020 (Cancer diagnoses data tables, table 2). Accessed December 2022	
2.	Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications	• The indication is for [redacted] • The vast majority of IDH1 mutations in cholangiocarcinoma are at the R132 residue (~97%)[1] • Approximately 13% of intrahepatic and 1% of extrahepatic cholangiocarcinomas have IDH1 mutations.[1] • This equates to an annual incidence of IDH1 positive cholangiocarcinoma of approximately 260. [(1945 * 0.13) + (672 * 0.01)] – See previous section. • Assuming a one year survival of 27.5% (midpoint between female and male survival) and a 5 year survival of 5%[2], (although it is unclear whether these figures are with current treatment options) it is	Met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
		unlikely that the incidence of IDH1 positive cholangiocarcinoma will exceed 300. • If there are assumed to be 260 incident cases each year then it is unlikely that more than 300 people would be eligible for ivosidenib at any one time. • Company estimates that between 55% and 90% of those with IDH1 positive cholangiocarcinoma would be eligible for chemotherapy regimens. • Even assuming no deaths in a year and that all people progressed on chemotherapy the population for the indication would likely not exceed 300. • The company have stated they have other indications for this drug in the USA (which if granted in GB may take the total population above 500) however they have stated they do not intend to pursue a licence for these in GB. 1. Boscoe. AN, Rolland. C, Kelley. RK, Frequency and prognostic significance of isocitrate dehydrogenase 1 mutations in cholangiocarcinoma: a systematic literature review. Journal of Gastrointestinal Oncology. Accessed December 2022 2. Cancer Research UK – Bile duct cancer survival– Accessed December 2022	
3.	The very rare disease for which the technology is indicated	• All stage one year survival in 2015 for bile duct cancer was around 30% and 25% for men and women respectively.[1] • 5 Year survival was 5% for both sexes	Met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
	significantly shortens life or severely impairs quality of life	• Most cases are diagnosed at the advanced stage where the cancer has already spread beyond the bile duct.[1] 1. Cancer Research UK – Survival for bile duct cancer – Accessed December 2022	
4.	There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.	• The other therapy for cholangiocarcinoma (beyond surgery) included on the NICE draft scope or raised in the consultation comments responses was chemotherapy consisting of modified FOLFOX regimens (folinic acid, fluorouracil and oxaliplatin) • Pemigatinib is recommended in NICE technology appraisal guidance 722 for cholangiocarcinoma where an FGFR2 mutation is present, however overlap between this mutation and IDH1 mutations is reportedly minimal.[1] • In TA722, the comparators were mFOLFOX and active symptom control, or active symptom control alone. • mFOLFOX is suitable for most, but not all people with cholangiocarcinoma who have had at least one prior systemic therapy. The clinical benefit from mFOLFOX may be limited but this is uncertain, and that the additional benefit of Ivosidenib over existing treatment options is unclear. • However as there are treatment options for the substantial proportion of people for this indication, this criterion is not considered as clearly met. 1. Saborowski. A, Lehman. U and Vogel. A – FGFR inhibitors in cholangiocarcinoma: what's now and what's next – Accessed December 2022	Not met