National Institute for Health and Care Excellence

Health Technology Evaluation

Elafibranor for treating primary biliary cholangitis ID6331
Response to stakeholder organisation comments on the draft remit and draft scope

Please note: Comments received in the course of consultations carried out by NICE are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the submissions that NICE has received, and are not endorsed by NICE, its officers or advisory committees.

Comment 1: the draft remit and proposed process

Section	Stakeholder	Comments [sic]	Action
Appropriateness of an evaluation and proposed evaluation route	Ipsen Limited	The topic and proposed evaluation route are appropriate.	Thank you for your comment. No action needed.
Wording	Ipsen Limited	Yes, the remit is appropriate.	Thank you for your comment. No action needed.
Timing Issues	Ipsen Limited	Despite the availability of Obeticholic acid (OCA) as a second line option, not all patients receive it and there remains an undertreatment of patients who have an inadequate response to Ursodeoxycholic acid (UDCA) or cannot tolerate UDCA.[1] Therefore, timely assessment of elafibranor in line with its marketing authorisation timelines is necessary. Reference:	Thank you for your comment. No action needed.

Section	Stakeholder	Comments [sic]	Action
		1. Abbas, N. et al. Critical shortfalls in the management of PBC: Results of a UK-wide, population-based evaluation of care delivery. JHEP Rep 6, 100931 (2024).	
Additional comments on the draft remit	Ipsen Limited	None	Thank you. No action needed.

Comment 2: the draft scope

Section	Consultee/ Commentator	Comments [sic]	Action
Background information	Ipsen Limited	Accurate and complete	Thank you for your comment. No action needed.
Population	Ipsen Limited	Yes, the population is appropriately defined.	Thank you for your comment. No action needed.
Subgroups	Ipsen Limited	No, there are no subgroups of the population in whom elafibranor alone or in combination with UDCA is more clinically effective.	Thank you for your comment. No action needed.

Section	Consultee/ Commentator	Comments [sic]	Action
Comparators	Ipsen Limited	Yes, the comparators listed are the relevant comparators for this appraisal. However, it should be made clearer that elafibranor will be used in combination with (i.e., added to) UDCA for patients who have an inadequate response to UDCA. Therefore, the comparators in patients who have an inadequate response to UDCA should be listed as: • OCA in combination with UDCA • UDCA with no additional treatment The comparators for patients who are unable to tolerate UDCA should be listed as: • OCA monotherapy • No treatment Please note however that only approximately 5% of patients are unable to tolerate UDCA, as reflected in the proportions of patients in the OCA and elafibranor trials.[1,2,3,4] References: 1. Obeticholic acid for treating primary biliary cholangitis | Guidance | NICE. https://www.nice.org.uk/guidance/ta443. 2. Nevens, F. et al. A Placebo-Controlled Trial of Obeticholic Acid in Primary Biliary Cholangitis. N Engl J Med 375, 631–643 (2016). 3. Invernizzi, P. et al. Primary Biliary Cholangitis: advances in management and treatment of the disease. Dig Liver Dis 49, 841–846 (2017). 4. Kowdley, K. V. et al. Efficacy and Safety of Elafibranor in Primary Biliary Cholangitis. N Engl J Med (2023) doi:10.1056/NEJMoa2306185.	Thank you for your comment. The comparators have been clarified in the final scope.

Section	Consultee/ Commentator	Comments [sic]	Action
Outcomes	Ipsen Limited	Yes, the outcomes listed are appropriate.	Thank you for your comment. No action needed.
Equality	Ipsen Limited	As PBC is a rare disease, it is essential that patients have the same opportunity to receive new therapies. PBC disproportionately affects women compared to men, with a nearly tenfold higher incidence in women.	Thank you for your comment. The committee will consider potential equality issues during the appraisal.
Other considerations	Ipsen Limited	N/A	Thank you. No action needed.
Questions for consultation	Ipsen Limited	*Where do you consider elafibranor will fit into the existing care pathway for PBC?* Elafibranor would be a second-line treatment option added to UDCA in adult patients who have an inadequate response to UDCA, or as monotherapy in adults who are unable to tolerate UDCA. *Are ursodeoxycholic acid and obeticholic acid the only relevant comparators?* Yes, UDCA and OCA are the relevant comparator treatments for PBC. *Is best supportive care a relevant comparator? If so, how should it be defined?* No *Would elafibranor be a candidate for managed access?*	Thank you for your comments. No action is needed.

Section	Consultee/ Commentator	Comments [sic]	Action
		No. *Do you consider elafibranor to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?* Yes, elafibranor is a simple once daily dosing regimen which does not require dose titration, whereas UDCA is dosed on mg/kg basis taken as multiple tablets in divided doses per day and OCA requires dose titration depending on response and tolerance to the regimen. *Do you consider that the use of elafibranor can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?* Yes, elafibranor is a simple once daily dosing regimen which does not require dose titration or adjustment, so the simpler administration may benefit patients. *Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits*. The dosing schedule of elafibranor is per the summary of product characteristics.	
Additional comments on the draft scope	Ipsen Limited	None	Thank you. No action needed.

The following stakeholders indicated that they had no comments on the draft remit and/or the draft scope

British Liver Trust