Predicted lifetime health care resource use costs associated with the treatment of patients with primary biliary cholangitis from a UK payer perspective

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BACKGROUND

- Primary biliary cholangitis (PBC) is a rare, autoimmune, chronic, cholestatic liver disease characterized by the destruction of small intrahepatic bile ducts. Uncontrolled, the disease can progress to other complications including cirrhosis, liver failure and death.[1]
- Ursodeoxycholic acid (UDCA) is the only treatment recommended in the first-line setting by European Association for the Study of the Liver (EASL).[1] Second-line treatment is limited to obeticholic acid (OCA), as recommended by both EASL and the American Association for the Study of Liver Disease (AASLD),[1,2] though this guidance predates and, therefore, does not reflect the availability of newly licensed second-line treatments like elafibranor and seladelpar. Additionally, due to safety concerns, marketing authorisation for OCA has since been revoked and restricted by the European Medicines Agency (EMA) and Federal Drug Administration (FDA), respectively.*
- PBC treatment carries substantial medical and non-medical costs that increase with disease progression.[3] Liver transplantation in advanced stages is a major cost driver as it is a complex surgical procedure associated with extensive health care resource utilisation (HCRU) for the procedure, pre- and post-operative care.[3] Thus, disease modifying drugs that delay disease progression may help reduce the cost burden on health care payers.

OBJECTIVE

- To assess the costs associated with the HCRU of second-line treatment options for PBC from a UK health care payer perspective.

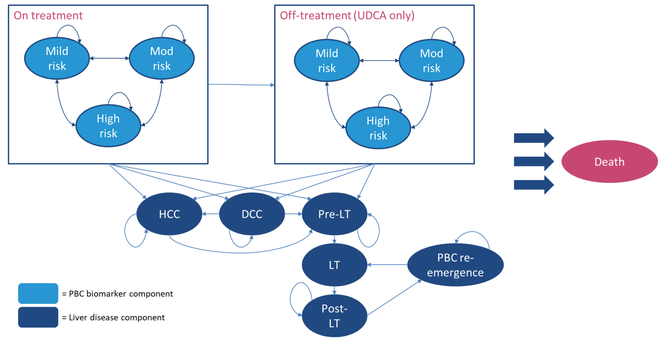
CONCLUSIONS

- In the model, patients treated with elafibranor incur the lowest total HCRU costs compared with those treated with OCA or UDCA. This is due to better outcomes for disease progression and consequent delay of transitioning to the more costly late-stage health states.
- Elafibranor for the second-line treatment of patients with PBC may reduce the cost burden for health care payers.

METHODS

- A 10-health state Markov cohort model was used to model the progression of PBC. The model structure was consistent with economic models identified in a systematic literature review and was validated with clinical experts.[4, 5-8]
- The model consists of a PBC biomarker component which stratified patients by risk of disease progression (mild-, moderate- or high-risk) based on alkaline phosphatase, total bilirubin, and liver stiffness and a second component, of liver disease, comprised of patients with progressed disease (Figure 1).
- The model included patients with PBC in the second-line setting (i.e., who had an inadequate response to or unacceptable side effects with UDCA).
- A cycle length of three months was used over a lifetime time horizon to estimate the costs and outcomes of second-line treatment of PBC. Inclusion of UDCA reflects patients who do not receive further treatment after inadequate response.
- Transitions for elafibranor and UDCA in the PBC biomarker component were based on the ELATIVE trial (NCT04526665); for OCA, an indirect comparison was used. Literature informed transitions from the high-risk state to the liver disease component as well as transitions within the liver disease component. Clinical expert opinion informed transitions from the moderate-risk state to the liver disease component. Patients in the mild-risk state were assumed unable to transition directly to the liver disease component.
- HCRU and costs, inflated to 2023, were sourced from previous National Institute for Health and Care Excellence (NICE) appraisals, national databases and literature (Table 1).[4, 9-11] An annual 3.5% discount rate was applied to both costs and outcomes.
- Health state costs within the model comprised only of the costs of managing and monitoring patients, including: inpatient visits, medical procedures, outpatient visits, blood tests, liver transplant-related resources and palliative care. Palliative care is considered only for patients who die in the decompensated cirrhosis (DCC) and hepatocellular carcinoma (HCC) health states.
- Treatment acquisition, adverse event and pruritus management costs were not included in the analyses.

Figure 1: Structure of the economic model



RESULTS

- In the model, elafibranor had the lowest total HCRU costs compared to OCA and UDCA (Table 2).
- Elafibranor also had the lowest HCRU costs across all health states in the model, except the mild- and moderate-risk health states (Table 3).
- Lower costs for elafibranor-treated patients were driven by fewer transitions to the higher-cost high-risk and progressed disease health states than OCA- or UDCA-treated patients, resulting in more time spent in the lower-cost mild- and moderate-risk health states. These results indicate better disease control for patients treated with elafibranor when compared to OCA and UDCA.
- Despite having the highest costs associated with the mild- and moderate-risk health states, elafibranor accrued the lowest HCRU costs within the PBC biomarker component of the model which included the mild-, moderate- and high-risk health states (Table 2).
- Elafibranor accrued the lowest HCRU costs associated with the liver disease component of the model as well as each individual health state within this component (DCC, HCC and liver transplant, Table 3). This reflects fewer and delayed cases of DCC, HCC and liver transplant compared to OCA and UDCA over the lifetime time horizon of the model.
- Additionally, elafibranor had the lowest HCRU costs for palliative care compared to OCA and UDCA (Table 2). This suggests improved survival outcomes in patients treated with elafibranor.

Table 1: Total HRCU costs per cycle per health state

Health state	Total HRCU cost per cycle (GBP)	Sources
Mild-risk	114.01	TA443[4]
Moderate-risk	165.36	TA443[4]
High-risk	2223.53	TA443[4], Wright *et al.* 2006[10]
DCC	4447.06	Wright *et al.* 2006[10]
HCC	3263.20	Wright *et al.* 2006[10]
Pre-LT	5659.62	HST17[9]
LT†	174847.35	HST17[9]
Post-LT	981.45	HST17[9], Rice *et al.* 2021[11]
PBC re-emergence	2223.53	TA443[4], Wright *et al.* 2006[10]
Death (DCC)*	11,649.71	Gola *et al.* 2015[12]
Death (HCC)*	9,407.98	TA666[13]

† All costs associated with the LT and first two years following the LT were applied in the cycle in which the LT occurred as a one-off cost since patients reside in the LT health state for one cycle only. * End of life costs were included for patients who die in health states where there is expected to be palliative care. This includes patients who die in the DCC and HCC health states.

Table 2: Total health state HRCU costs by model component

Model component	HRCU costs per model component per treatment option (GBP)		
	Elafibranor	OCA	UDCA
PBC biomarker	21,837.65	27,595.76	32,585.22
Liver disease	39,174.98	56,578.35	71,287.08
Death	2,991.06	4,113.37	4,679.80
Overall	64,003.69	88,287.48	108,552.10
Incremental overall costs of elafibranor vs comparator	-	-24,283.79	-44,548.41

Table 3: Breakdown of health state HRCU costs by treatment option

Health state	HRCU costs per health state per treatment option (GBP)		
	Elafibranor	OCA	UDCA
Mild-risk	3,056.54	1,060.84	173.93
Moderate-risk	2,315.68	2,951.17	1,974.88
High-risk	16,465.43	23,583.75	30,436.41
DCC	13,424.03	19,372.80	24,367.61
HCC	743.17	1,070.93	1,342.53
Pre-LT	5,511.99	7,944.56	9,966.17
LT	16,853.29	24,308.38	30,531.36
Post-LT	2,036.86	2,984.35	3,877.90
PBC re-emergence	605.64	897.33	1,201.50
Death	2,991.06	4,113.37	4,679.80
Total costs	64,003.69	88,287.48	108,552.10

Abbreviations AASLD: American Association for the Study of Liver Diseases; DCC: Decompensated cirrhosis; EASL: European Association for the Study of the Liver; FDA: Food and Drugs Administration; GDAC: Gastrointestinal Drugs Advisory Committee; GBP: Great British Pound; HCC: Hepatocellular carcinoma; HCRU: Health care resource use; LT: Liver transplant; Mod: Moderate; NICE: National Institute for Health and Care Excellence; OCA: Obeticholic acid; PBC: Primary biliary cholangitis; UDCA: Ursodeoxycholic acid.

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*As of November 27th, 2024, the EMA has revoked the conditional marketing authorisation for OCA. The Food and Drugs Administration's (FDA) Gastrointestinal Drugs Advisory Committee (GDAC) have also recommended revoking its licensing authorisation. Consistent with the GDAC's recommendation, the FDA have decided to decline the supplemental New Drug Application for OCA in November 2024.

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