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CONCLUSIONS

- The findings of this systematic literature review indicate that PBC patients are associated with lower HUVs compared to the general population or healthy controls
- PBC patients who experienced two or more symptoms, such as depression, pruritus, and sleep disturbances, had lower HUVs than PBC patients with pruritus
- These findings emphasize the importance of capturing symptom-specific and treatment-responsive HUVs to support health economic evaluations in PBC for healthcare decision-making

INTRODUCTION

- Primary biliary cholangitis (PBC) is a rare, progressive autoimmune liver disease that can lead to cirrhosis and liver failure
- PBC is often associated with range of debilitating symptoms which can significantly impact the patient’s quality of life (QoL)<sup>1</sup>
- Health utility values (HUVs), a critical parameter for economic evaluations (EE) and health technology assessments (HTA), remain limited and heterogeneous in PBC<sup>2</sup>
- Assessing symptom-specific and treatment-related utility values is crucial for informed healthcare decision making in PBC

OBJECTIVE

- This systematic literature review (SLR) aimed to identify and summarize the published global evidence on HUVs in adult patients with PBC

METHODS

- This review was conducted following the standard methodology outlined in the National Institute for Health and Care Excellence (NICE) and the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) guidelines
- Key biomedical databases such as EMBASE® and MEDLINE® were searched using relevant keywords for HUVs and PBC. English-language studies reporting outcomes specific to adult PBC populations were included from database inception until August 2024 to retrieve the latest evidence
- Data was collected using the inclusion/exclusion criteria guided by the PICOS approach (Figure 1). Relevant studies were selected based on a two-step process: (1) title/abstract screening and (2) full-text screening.
- Citations were screened in parallel by a human (reviewer-1) and an artificial intelligence-based tool (GPT-4.0) (reviewer-2), with any discrepancies resolved by a human subject matter expert (reviewer-3)

Figure 1: PICOS criteria for inclusion in the SLR

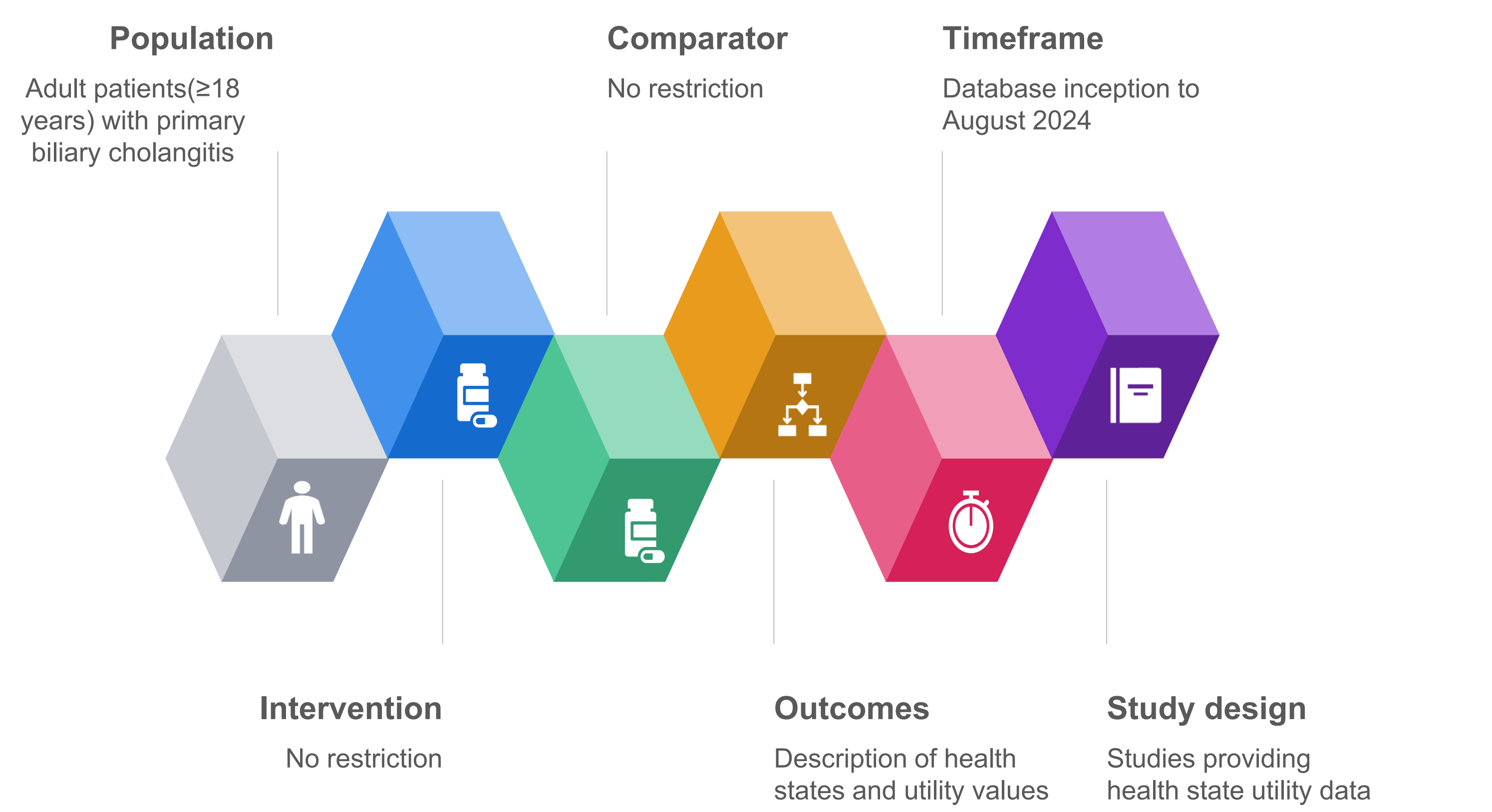
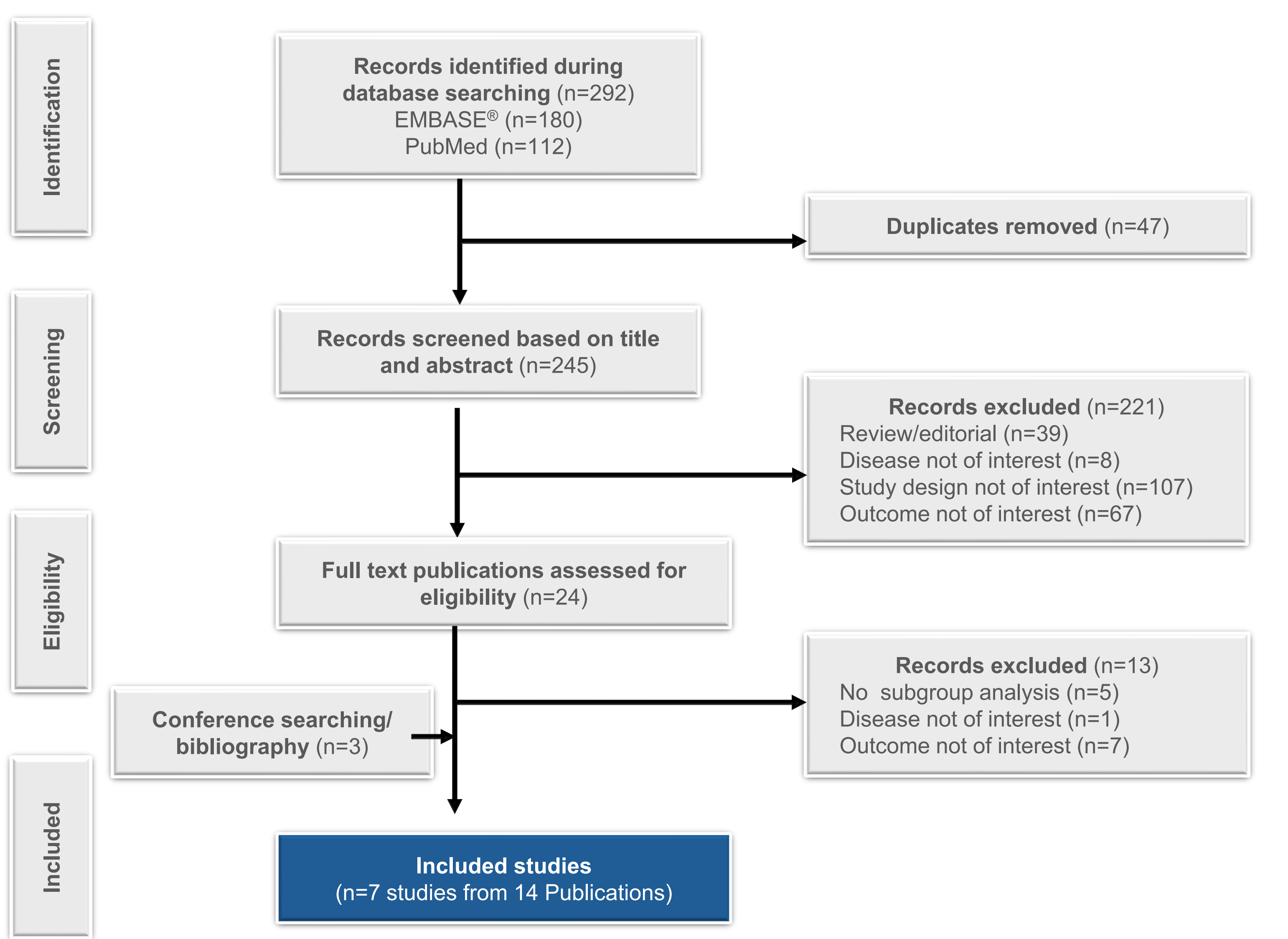


Figure 2: PRISMA diagram for the screening process



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PLAIN LANGUAGE SUMMARY

- This review identified studies reporting health utility values (HUVs) in adults with primary biliary cholangitis (PBC), a liver disease that affects daily functioning
- Compared to healthy individuals, adults with PBC were associated with lower HUVs, indicating worse health
- Furthermore, lower HUVs were reported in patients with two or more persistent symptoms, such as itching, poor sleep, and depression, compared to PBC patients with pruritus alone

RESULTS

- Of the 292 records screened, seven studies were included, which met the pre-specified eligibility criteria. **Figure 2** presents the PRISMA flow diagram
- Most of the included studies were published in peer-reviewed journal articles and were observational in design (**Figure 3**). The HUVs were assessed in all studies among patients with PBC. However, in the GLIMMER trial, the HUVs were evaluated among patients with pruritus due to PBC
- The majority of the studies reported female patients ranging from 84.00 to 100%, while the mean age ranged from 47.30 to 68.00 years (**Figure 4**)
- Overall, PBC patients demonstrated lower HUVs (0.28<sup>3</sup> to 0.87<sup>4</sup>) compared to the general population or healthy controls (0.82<sup>5</sup> to 0.95<sup>6</sup>) (**Table 1**)
- EuroQoL 5 Dimensions (EQ-5D):** Five of the seven studies utilized the EQ-5D-5L index, followed by one study that utilized the EQ-5D-3L index
  - Impact of symptoms:** Findings of the GLIMMER trial revealed that with an increase in the severity of pruritus, sleep interference, and depression, a decline in HUVs was observed. A significant decline in utility scores was evident among patients with depression, along with severe pruritus (Mean EQ-5D-5L index: 0.28 to 0.30), compared PBC patients with pruritus (0.69)<sup>3</sup> (**Figure 5**)
  - Liver transplant:** Longworth et al. reported markedly lower utility scores in transplant awaiting patients (Mean EQ-5D-5L index: 0.38 at listing to 0.50 at six months after listing) compared to the general United Kingdom population (0.82). A gradual improvement in utility values was observed post-transplant, from 0.58 at three months to 0.62 at 24 months post-transplant<sup>5</sup> (**Figure 6**)
  - Treatment with UDCA:** A study by Rice et al. demonstrated that patients receiving ursodeoxycholic acid (UDCA) therapy had better utility values compared with those not on UDCA (incremental utility: 0.03; 95% CI, 0-0.05)<sup>7</sup>
- Health Utilities Index (HUI):** One study utilized the HUI Mark-2 and 3 for measuring utility values. Findings of the study revealed lower utility values among PBC patients compared to healthy controls (Mean HUI-Mark 2 index: 0.81 vs 0.95). Patients scored better on the HUI-Mark 3 scale (0.90 on cognition to 0.99 on hearing subscales) compared to HUI-Mark 2 (0.83 on sensation to 0.99 on self-care subscales)<sup>6</sup>

Figure 3: Study characteristics

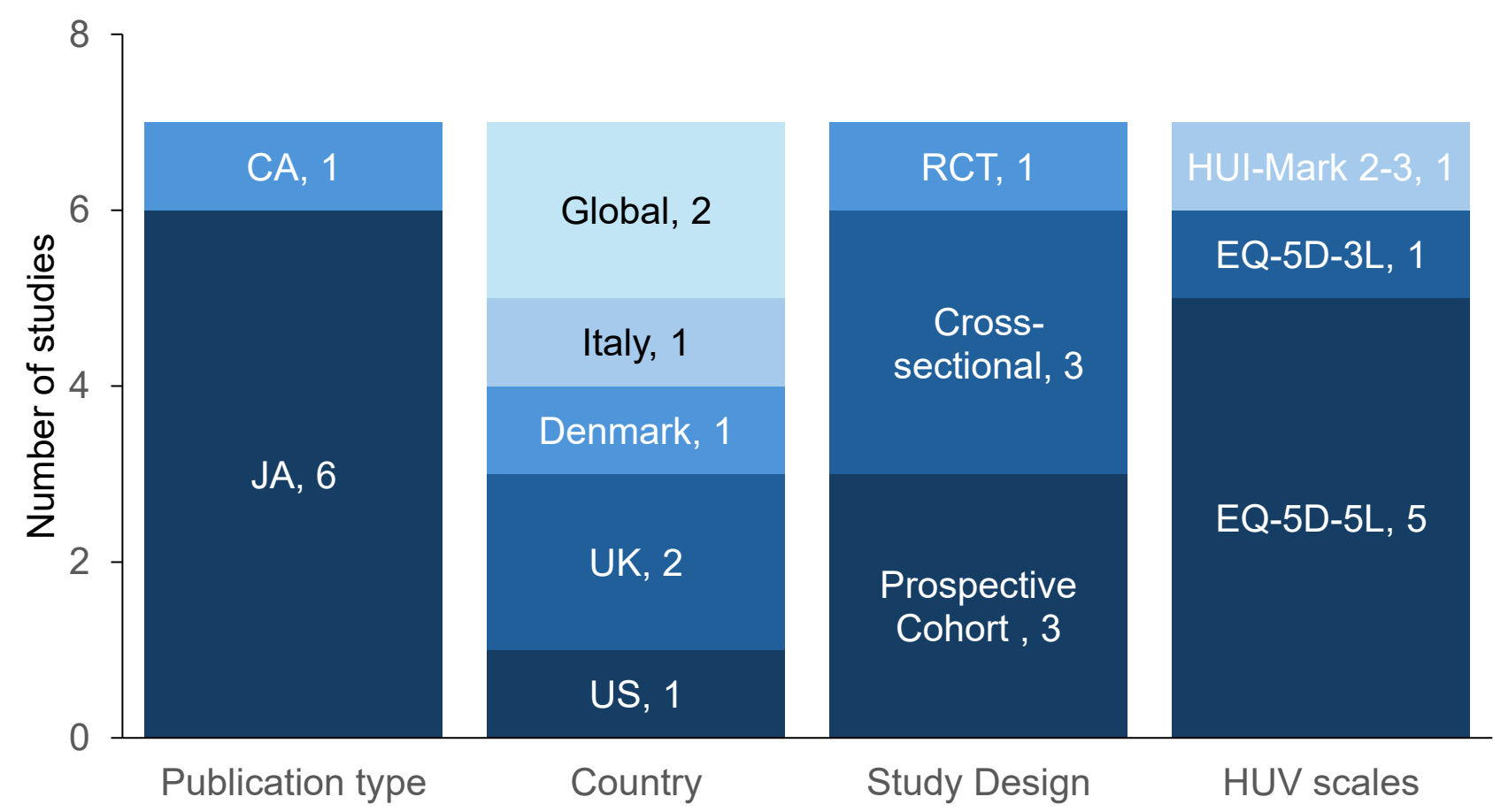
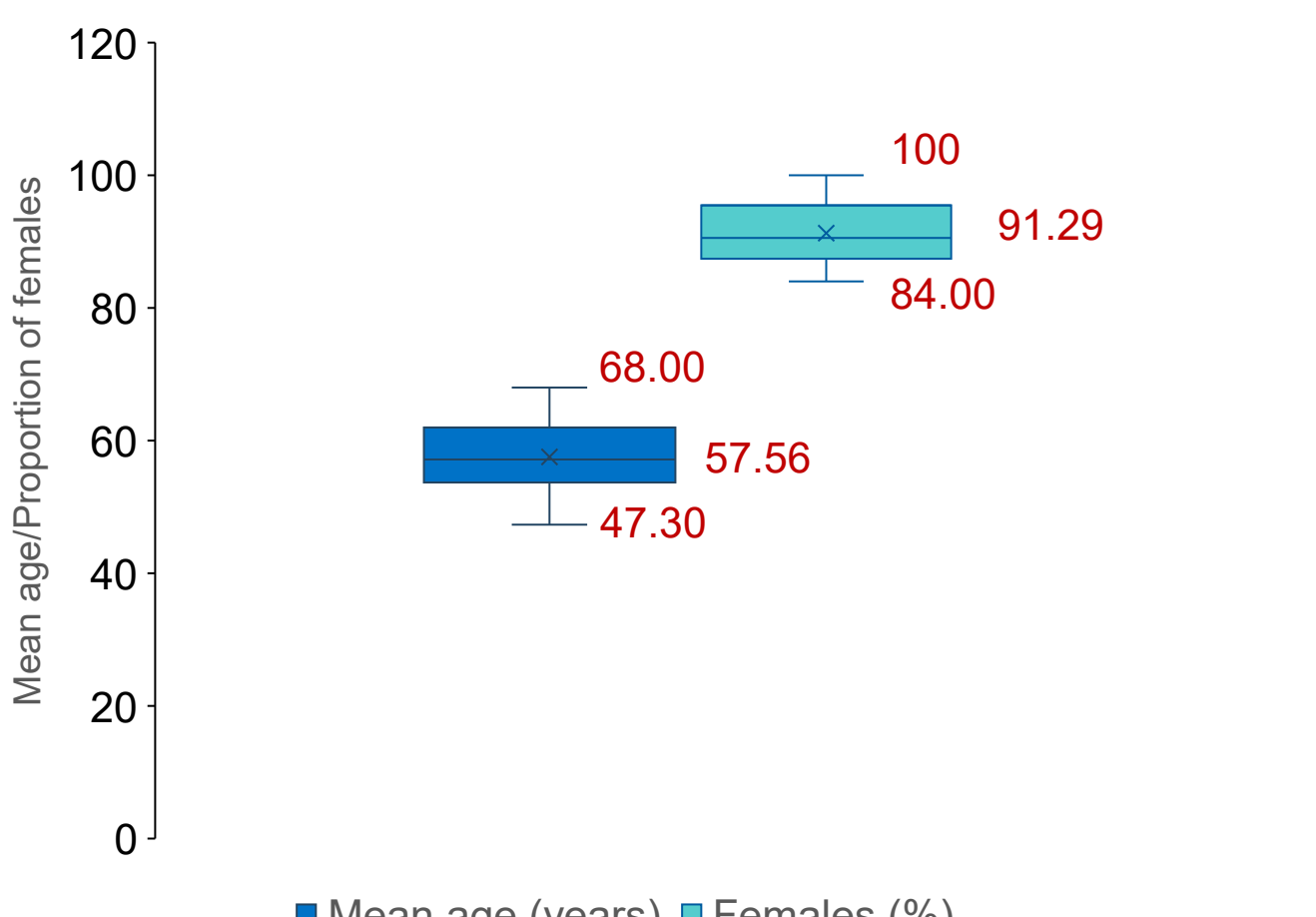


Figure 4: Population characteristics



CA: Conference abstract; EQ-5D: EuroQoL 5-Dimensions; HUI: Health utilities index; HUV: Health utility value; JA: Journal article; RCT: Randomized controlled trial

Table 1: Health utility values in Adult PBC Patients vs. Healthy Controls

Study name	Country	HUV scale	PBC (Mean scores)	Healthy controls (Mean scores)
Smith 2023 <sup>3</sup>	Global	EQ-5D-3L index	0.28 to 0.83	Not reported
Cortesi 2020 <sup>4</sup>	Italy	EQ-5D-3L index	0.87	0.91
Longworth <sup>5</sup>	UK	EQ-5D-5L index	0.38 to 62	0.82
Bondini 2007 <sup>6</sup>	United States	HUI-Mark 2 and 3	0.81	0.95
Rørdam 2024 <sup>8</sup>	Denmark	EQ-5D-5L index	0.79	Not reported
Wunsch 2023 <sup>9</sup>	Global	EQ-5D-5L index	0.73	Not reported

EQ-5D: EuroQoL 5-Dimensions; HUI: Health utilities index; HUV: Health utility value

Figure 5: EQ-5D-5L mean scores by symptom severity — GLIMMER trial (Smith 2023)<sup>3</sup>

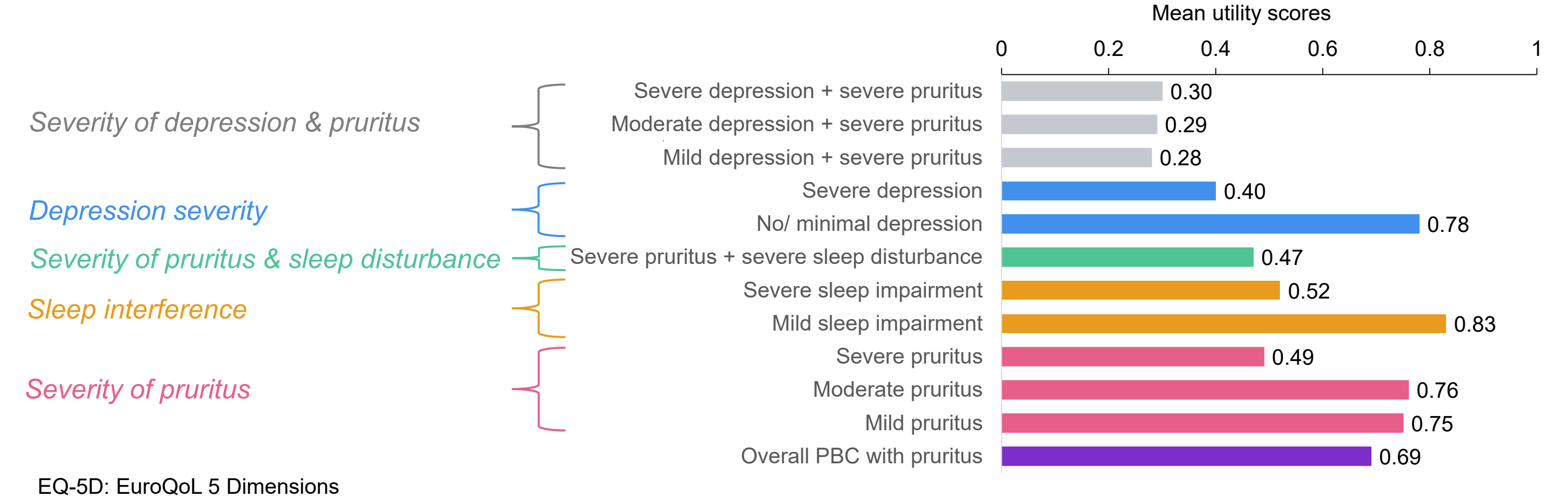
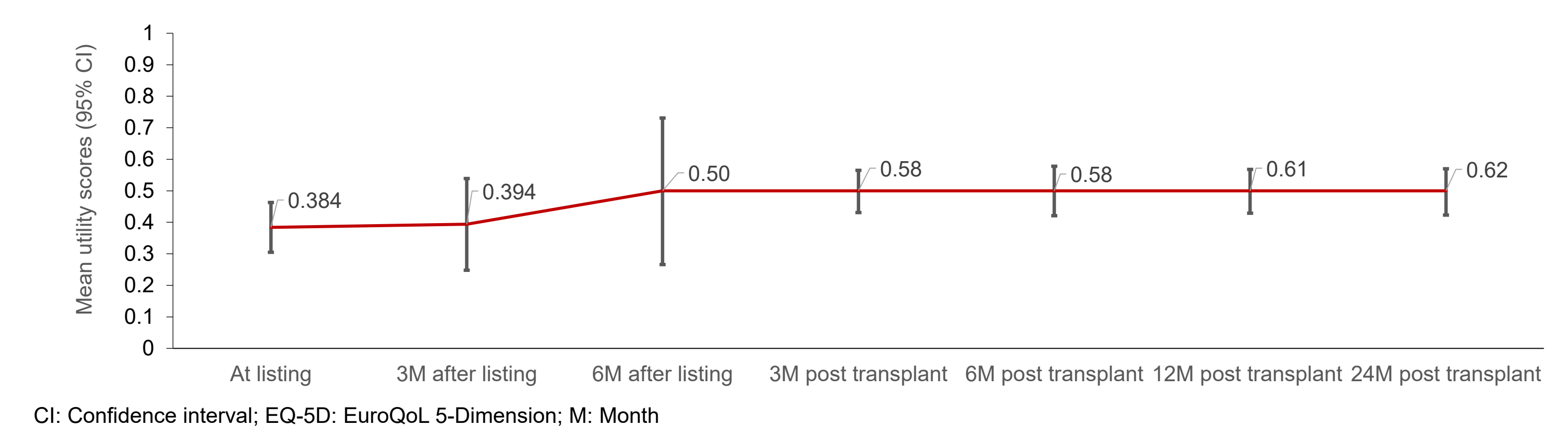


Figure 6: EQ-5D-5L mean score before and after liver transplant — (Longworth 2003)<sup>4</sup>



CI: Confidence interval; EQ-5D: EuroQoL 5-Dimension; M: Month

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Disclosures

DM, MR, CK, CB, MH, RT, and OE are employees of Gilead Sciences, Inc., SA, SS, and BS are employees of Pharmacovidence