

Dilip Makhija<sup>1</sup>, Marvin Rock<sup>1</sup>, Chong H Kim<sup>1</sup>, Caroline Burk<sup>1</sup>, Mirko von Hein<sup>2</sup>, Ryan Thaliffdeen<sup>1</sup>, Oskar Eklund<sup>3</sup>, Sumeet Attri<sup>4</sup>, Sukriti Sharma<sup>4</sup>, Barinder Singh<sup>4</sup>

<sup>1</sup>Gilead Sciences Inc., Foster City, CA, USA, <sup>2</sup>Gilead Sciences, London, United Kingdom, <sup>3</sup>Gilead Sciences AB, Solna, Sweden, <sup>4</sup>PharmacoEvidence, Mohali, India

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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

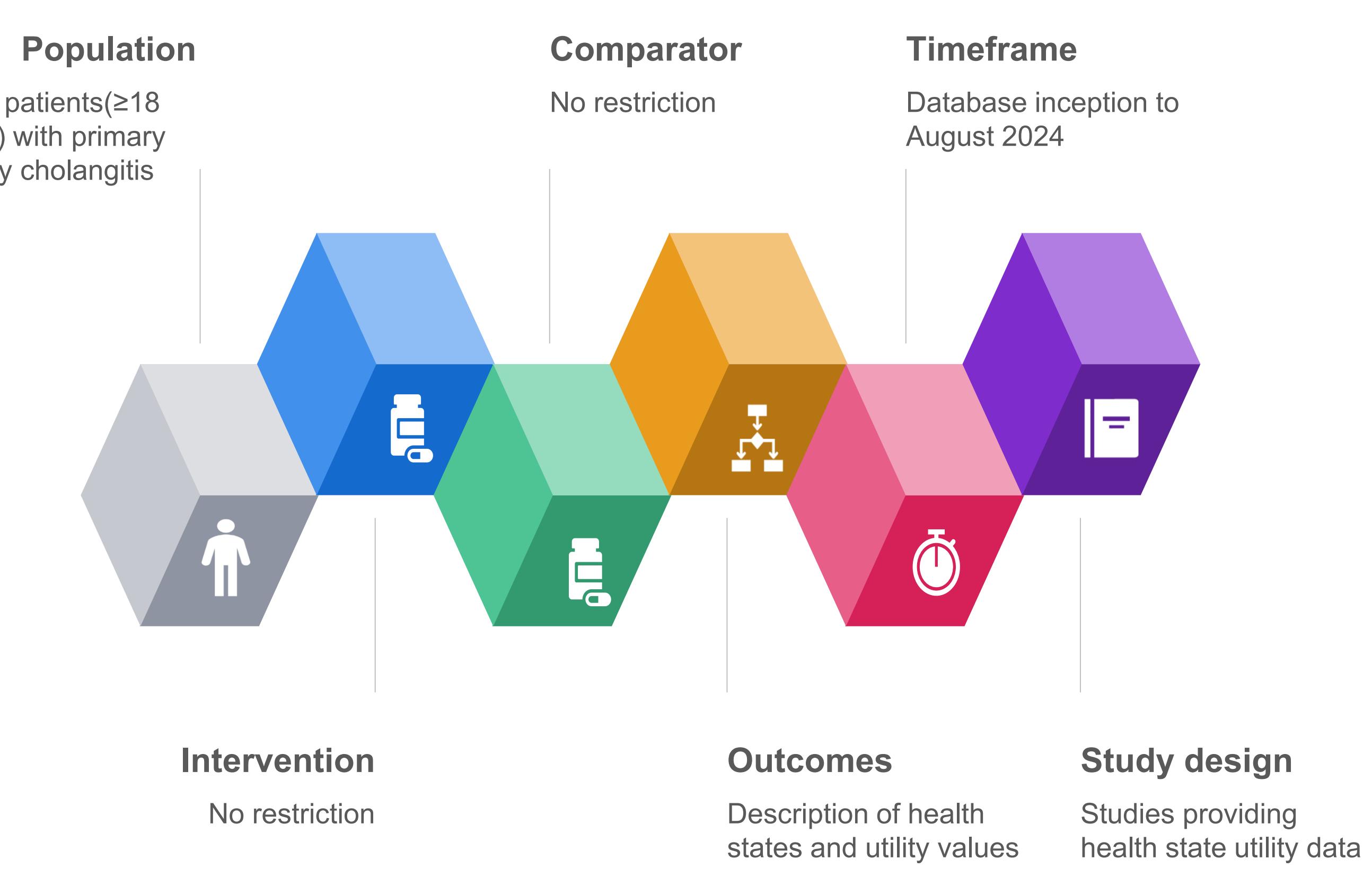
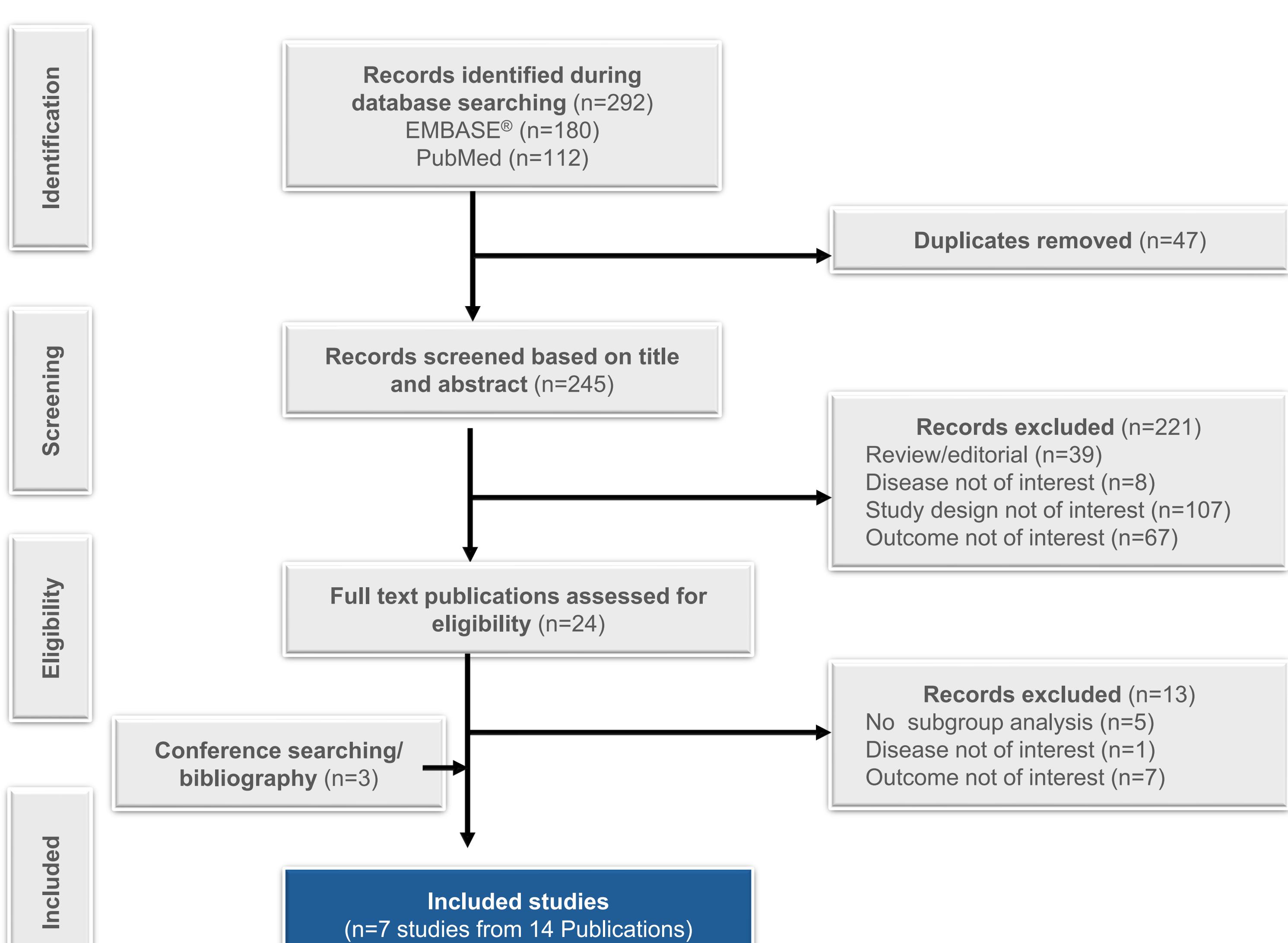


Figure 2: PRISMA diagram for the screening process



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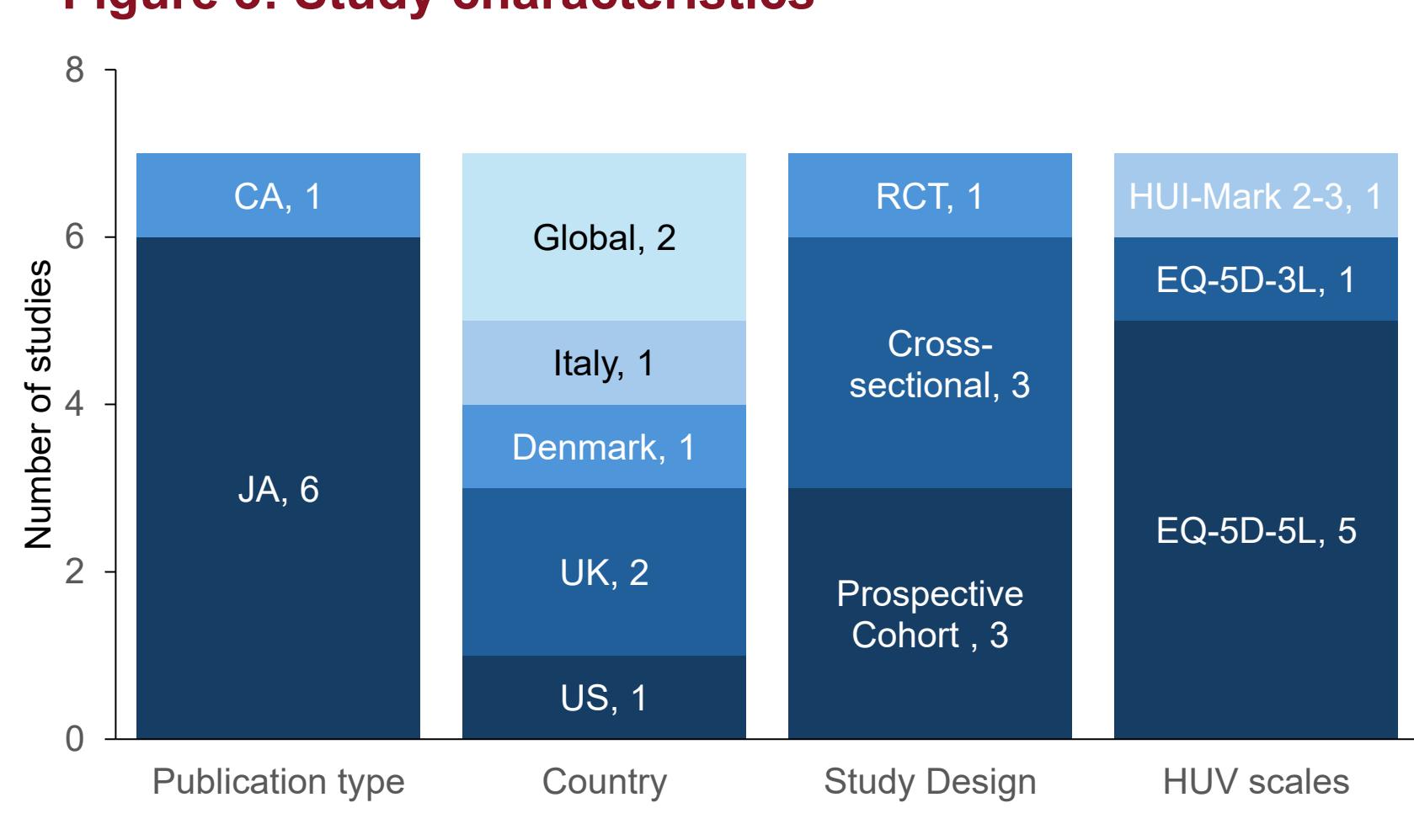
## PLAIN LANGUAGE SUMMARY

- 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>8</sup>

Figure 3: Study characteristics



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

Figure 4: Population characteristics

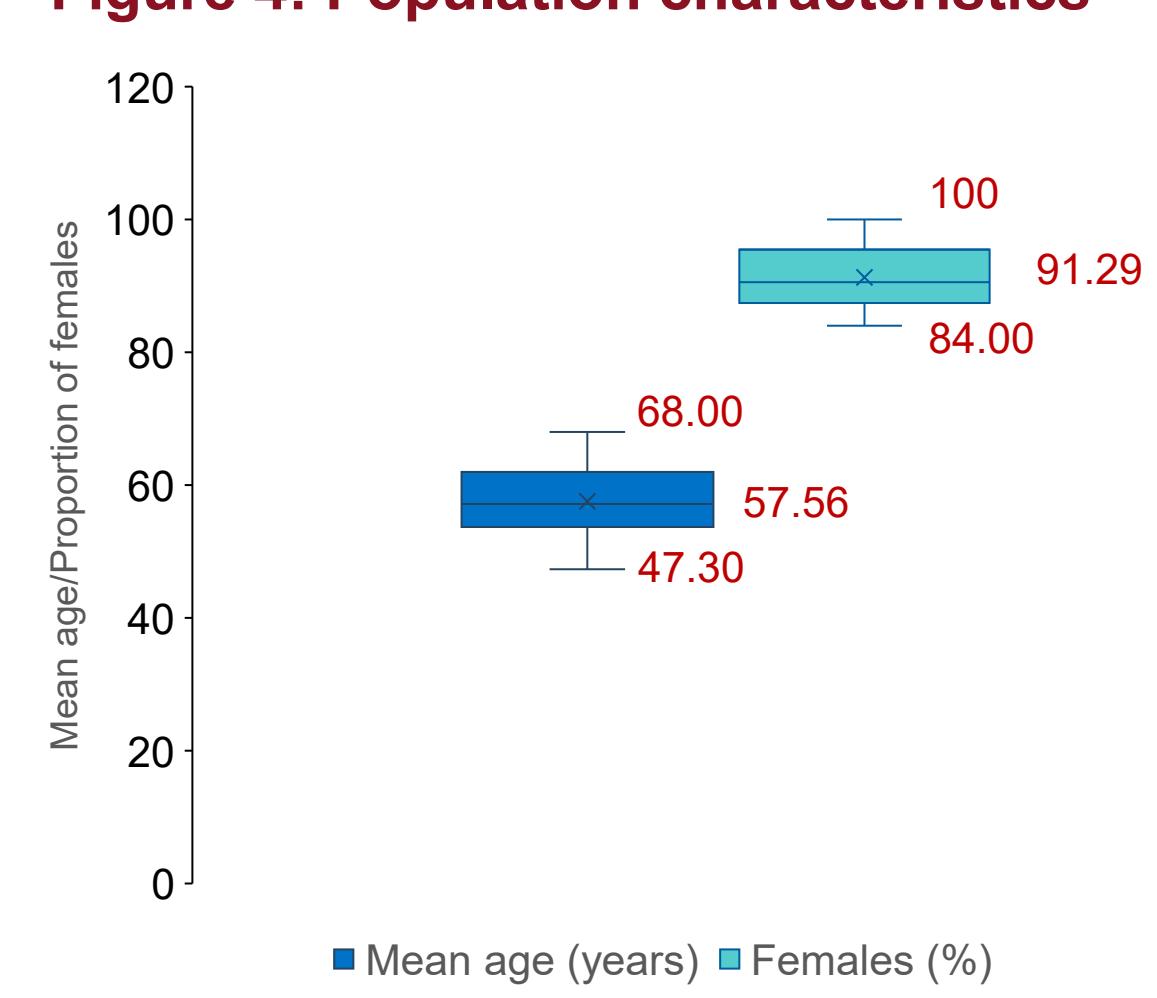


Table 1: Health utility values in Adult PBC Patients vs. Healthy Controls

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>

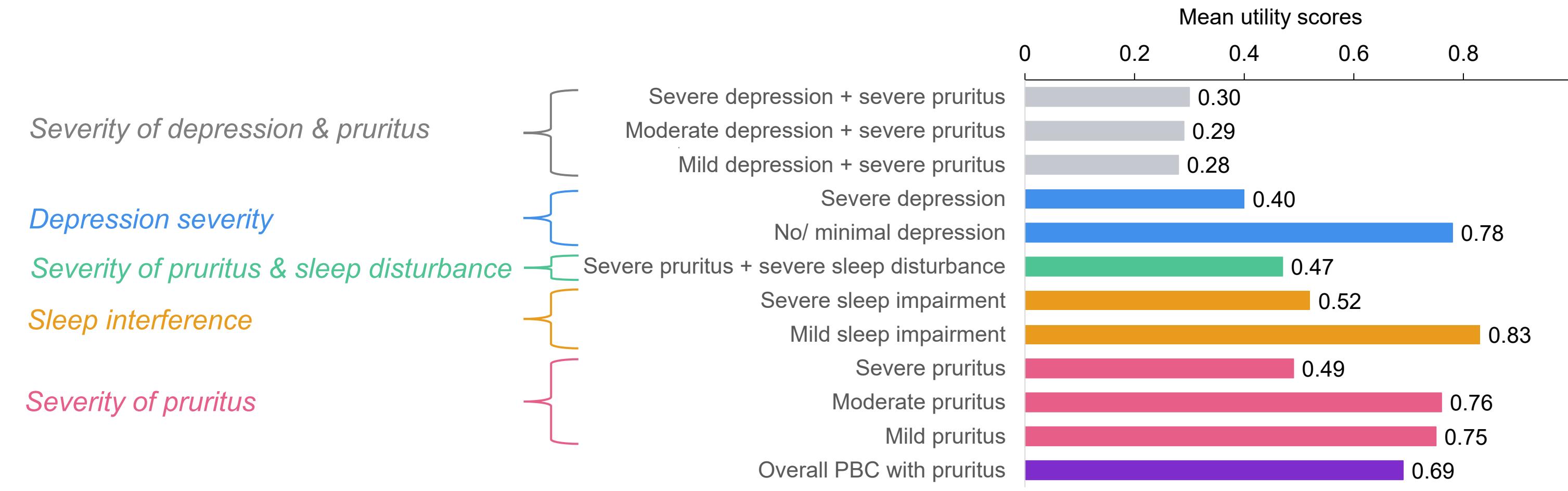
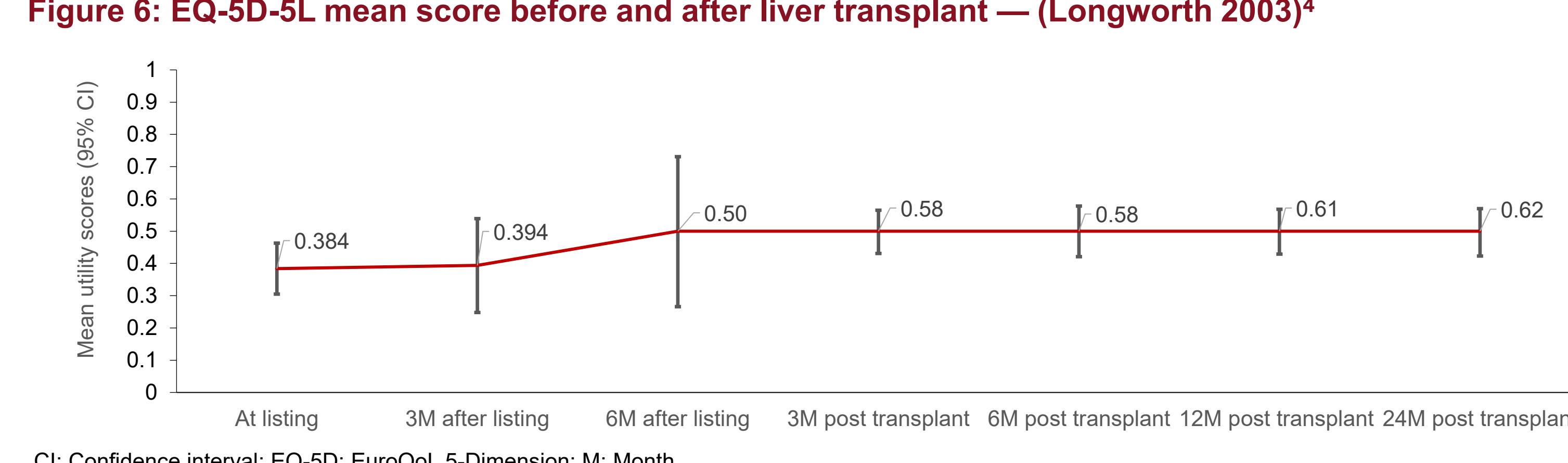


Figure 6: EQ-5D-5L mean score before and after liver transplant — (Longworth 2003)<sup>4</sup>



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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 PharmacoEvidence