

Systematic review of the cost effectiveness of newborn screening for severe combined immunodeficiency



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Background

Severe combined immunodeficiency (SCID) is a rare, inherited inborn error of immunity that is typically fatal in the absence of definitive treatment. This systematic review aimed to summarise international evidence on the cost effectiveness of newborn screening for SCID compared with detection based on family history or symptomatic presentation

Methods

A systematic search was carried out in in Medline (EBSCO), Embase (OVID) and the Cochrane Library from 1 January 2010 to 1 June 2022, supplemented by a grey literature search. The primary outcome of interest was the incremental cost-effectiveness ratio (ICER) of newborn screening for SCID compared with no screening (**Table 1**). All costs were converted to 2021 Irish euro. A protocol detailing the methods was published prior to the review.

The CHEC-list and ISPOR questionnaire were used to assess methodological quality and transferability of the economic evaluations. The reporting adhered to Preferred Reporting Items for Systematic reviews and Meta-Analyses criteria.

Table 1 Inclusion criteria

Population	Children
Intervention	Universal TREC-based newborn screening for SCID
Comparator	No newborn screening for SCID (with identification based on risk-based detection at birth or clinical presentation)
Outcome	ICER (for example, per life-year gained or quality-adjusted life-year) or NMB
Study design	Full economic evaluations: • Cost-utility analysis • Cost-effectiveness analysis

Key: ICER – incremental cost effectiveness ratio; NMB – net monetary benefit; TREC – T-cell receptor excision circles.

Results

Eleven studies, representing ten unique models, were included in the review. Of these, three performed a cost-utility analysis (CUA), three performed a cost-effectiveness analysis (CEA), and four presented results for both a CUA and a CEA. Eight studies adopted a healthcare system perspective, one a societal perspective, and one adopted both perspectives in separate analyses.

For studies reporting costs per life-years (LYs) gained, adjusted ICERs ranged from €14,027 to €217,657/LY (**Figure 1**). For the studies reporting costs per quality-adjusted LYs (QALYs) gained, the adjusted ICERs ranged from €14,549 to €83,670/QALY (**Figure 2**).

Most studies reported that the models were sensitive to variations in a number of key variables, including: test specificity, incidence of SCID, screening and diagnostic test costs, cost of treatment, and survival post-treatment.

Key findings

Results indicate that newborn screening for SCID may be a cost-effective intervention, compared with no screening, at willingness-to-pay thresholds between €20,000-€45,000/QALY. However, there is a large amount of uncertainty in terms of the model inputs and, thus, outcomes.

Figure 1 Adjusted ICERs per LY gained from model-based studies (based on adjustments to 2021 Irish Euro)

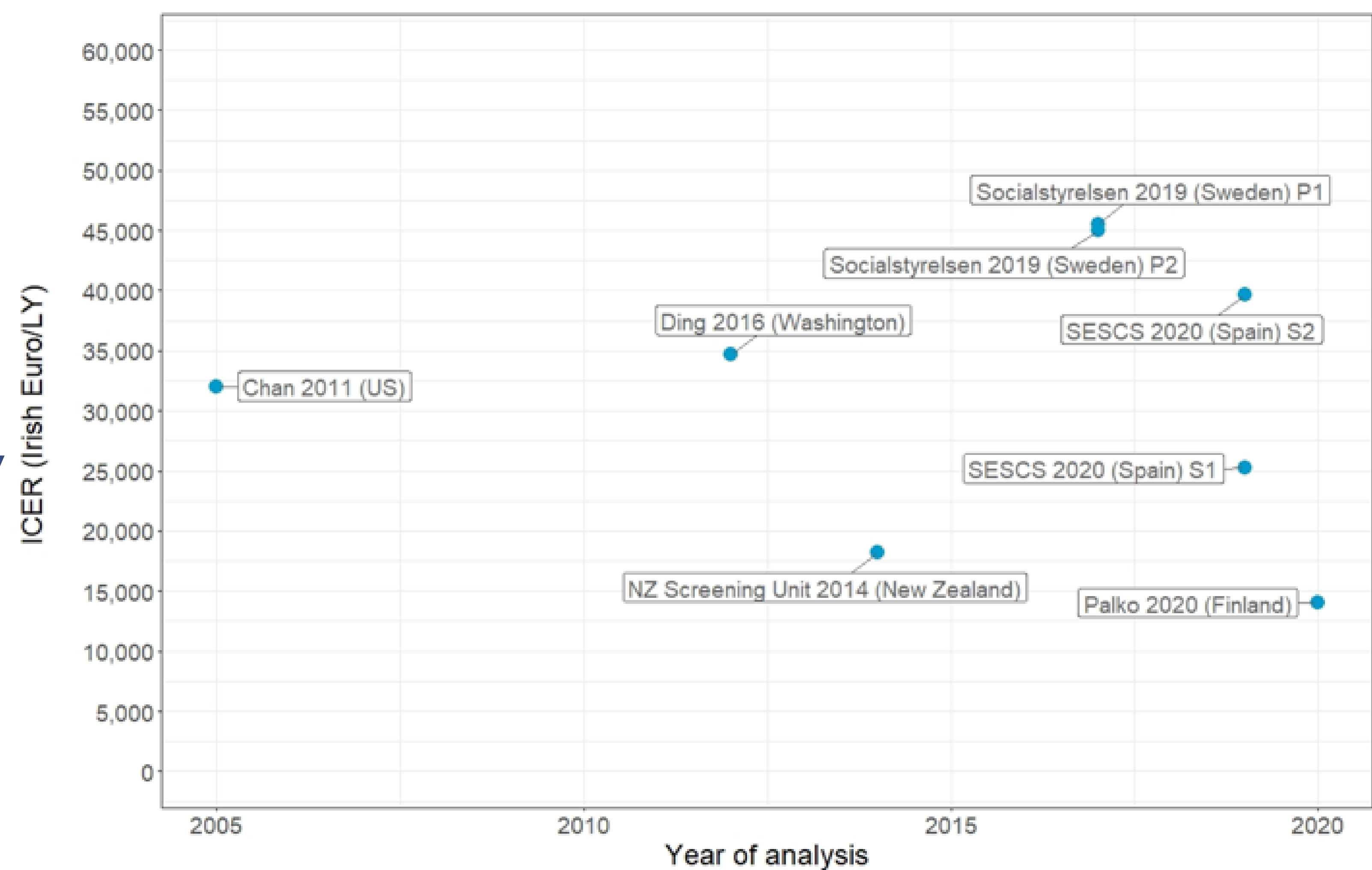
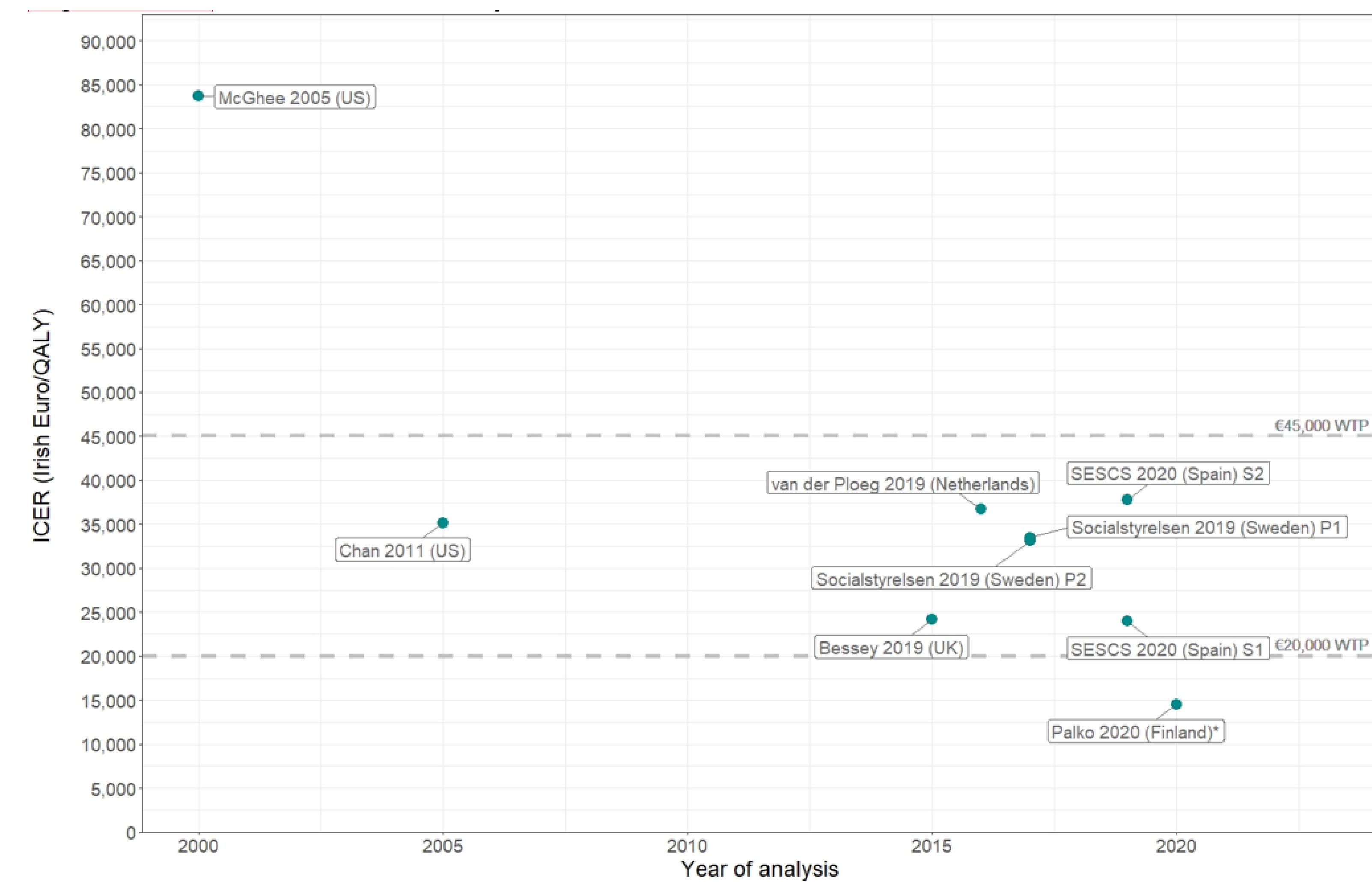


Figure 2 Adjusted ICERs per QALY gained from model-based studies (based on adjustments to 2021 Irish Euro)



Key: ICER – incremental cost effectiveness ratio, LY – life year, QALY - quality-adjusted life year, WTP - willingness-to-pay.

The study from Finland calculated ICERs for QALYs in sensitivity analyses, however this was not part of the base case analysis.

Multiple perspectives were presented from Sweden with P1 the being healthcare system perspective, P2 the being societal perspective.

Multiple base cases were presented for the study from Spain with highest and lowest values presented here (S1 – situation in which the adjusted unit cost of the screening test is €5.10 and the incidence is 1:50,000; S2 – situation in which the adjusted unit cost of the screening test is €7.65 and the incidence is 1:60,000).

Scan for more information

