Could early scientific advice have improved outcomes in cost-effectiveness markets?

George Binkley, Malgorzata Barchanska, James Campbell, Erika Turkstra

Parexel® International, London, UK

erika.turkstra@parexel.com

Background

- Early scientific advice (ESA) can assist with the development of the evidence package to support health technology assessment (HTA) submissions. We conducted a study to understand if ESA might have improved outcomes in “cost-effectiveness” markets.

Methods

- All negative appraisals published in England (NICE), Scotland (SMC), Canada (CADTH), and Australia (PBAC) from 1st January 2021 to 31st May 2022 were retrieved and analysed. Outcomes from re-submissions overrode the respective original appraisals.
- Appraisals were analyzed based on the negative feedback to determine whether an ESA could have improved the final assessment outcome.
- Whenever a negative outcome was driven by inappropriate study design or comparator, it was assumed that ESA could have been beneficial. In other cases (i.e., negative feedback focused on clinical value, cost-effectiveness or budget impact) the impact of potentially conducted ESA was assessed as low. The likelihood of receiving better HTA assessment outcome if an ESA had been conducted was grouped into the five categories („yes”, „probably”, „potentially”, „unlikely” and „no”).

Results

- Across the four markets, 50 negative appraisals were identified in separate technologies/ indications. Nine negative appraisals were identified in England, 10 in Canada and 10 in Scotland, and 27 in Australia, with 43 published reports.
- Among the identified negative appraisals, oncology drugs had the largest share of assessed products (48%), followed by endocrinology (16%) and neurology products (12%).
- Issues concerning cost-effectiveness (98%), comparative-effectiveness (65%), and trial design (47%) were most commonly cited as drivers for negative appraisals. Comparative effectiveness and trial design were cited as drivers in most (80%) of the published negative appraisals in Canada. Trial design was also commonly cited as a driver of negative appraisals in Scotland (70%) (Figure 1).
- Almost half (49%) of published submissions were considered as candidates that could have potential benefits from ESA (Table 1, Figure 2).
  - Of these submissions, trial design (76%), comparative effectiveness (62%), comparator (48%), and clinical value (48%) were cited as reasons for the negative appraisals.

Figure 1: Drivers of the negative HTA outcome shown as proportion of decisions (*more than one reason could be provided per one apparisal)*

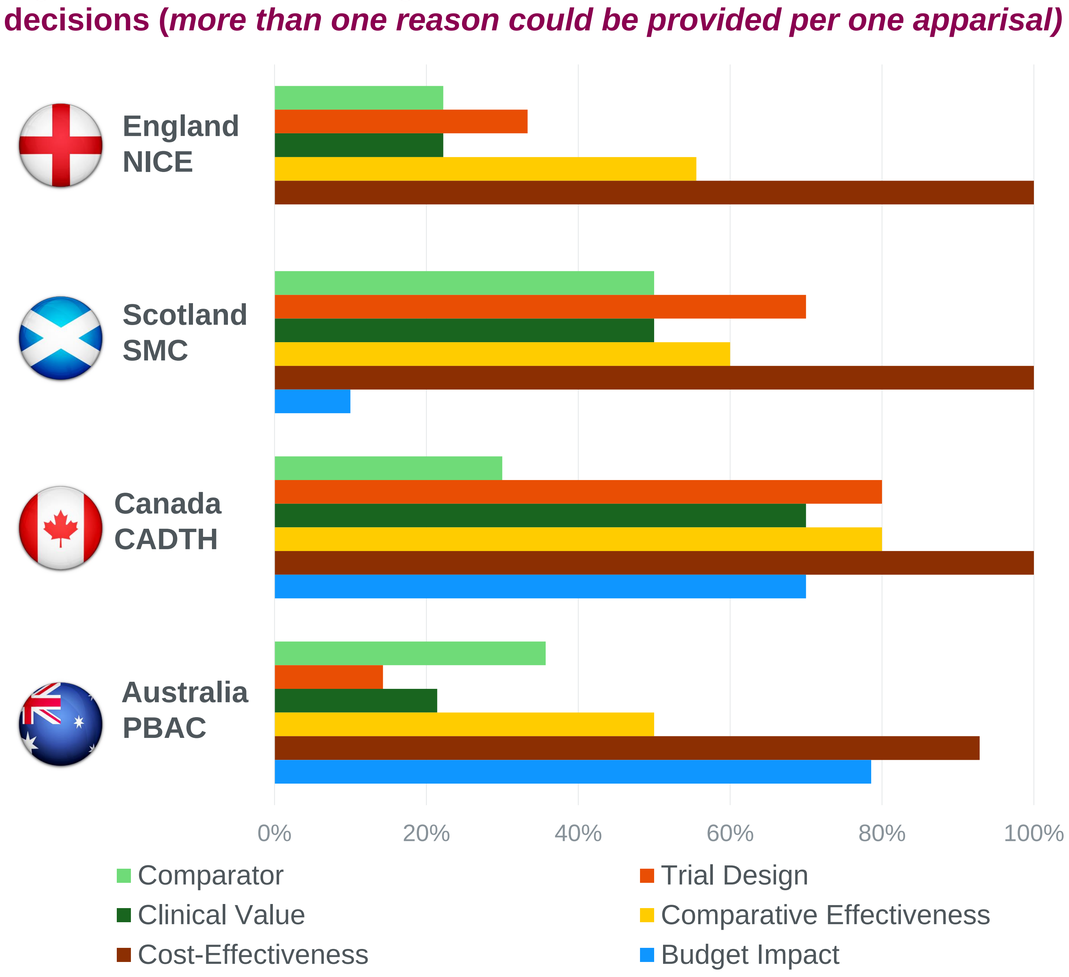
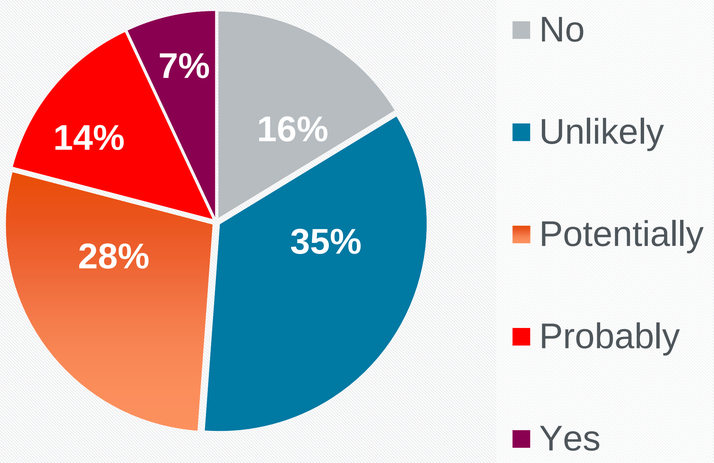


Table 1: Appraisals that could have benefited from ESA (%), by country

Appraisals that could have benefited from ESA (%)					
	No	Unlikely	Potentially	Probably	Yes
England (NICE)	0%	67%	22%	11%	0%
Scotland (SMC)	0%	30%	20%	30%	20%
Canada (CADTH)	0%	40%	40%	10%	10%
Australia (PBAC)	50%	14%	29%	7%	0%
Total (weighted)	**16%**	**35%**	**28%**	**14%**	**7%**

Figure 2: Appraisals that could have benefited from ESA (%), overall



Conclusions

- Identified negative appraisals were mostly driven by cost and comparative-effectiveness and trial design concerns. Based on the reasons for the rejection of submission and the areas of criticism indicated by HTA agencies, we concluded that almost half of the submissions with the negative HTA outcome could have potentially benefit from conducting an ESA.

Limitations

- The assessment of the magnitude of potential benefit from ESA was based solely on internal expertise. The absence of an ESA conducted was an assumption in the view of negative HTA outcome.
- The COVID-19 pandemic ongoing at the time of the evaluation period could potentially impact the acceptance of HTA submissions, which may limit the generalizability of the findings.