Characterization of the Evidence Supporting the Relative Effectiveness Evaluations in Health Technologies Assessment: A Review

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INTRODUCTION

Relative effectiveness assessment is a core step of HTA and aims to identify the benefit of a new healthcare intervention. [1] The procedure is based on an evaluation framework, which defines the candidate population, the comparators used in clinical practice, and the outcome measures of interest. [2] A comprehensive evidence base is fundamental to inform the evaluation and may consider various types of studies. [1] This study aims to the characterize the evidence supporting the relative effectiveness assessments of HTA procedures.

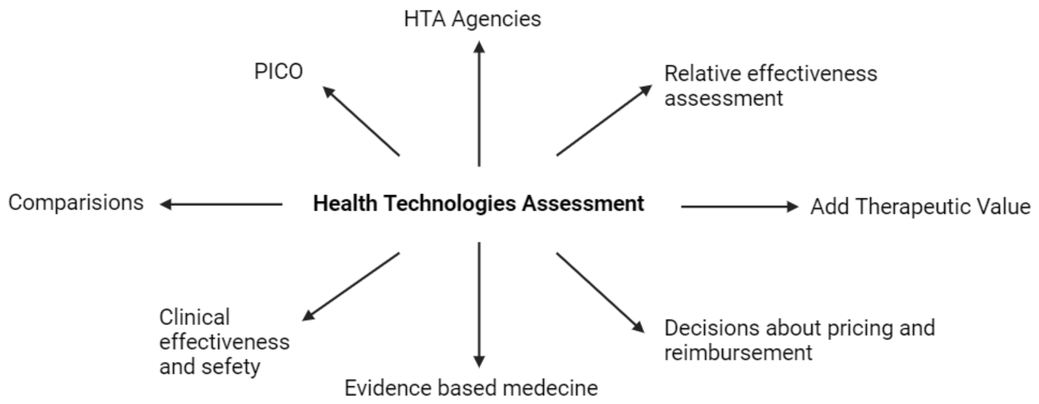

Figure 1 - Health Technologies Assessment.

METHODS

Relative effectiveness assessments produced between 2018 and 2021 by 6 European HTA bodies (NICE [England and Wales], SMC [Scotland], HAS [France], AEMPS [Spain], SiNATS [Portugal] and IQWIG [Germany]) were considered for inclusion. Data on the pharmacological intervention, comparators and data sources was retrieved. Direct evidence reflects evaluations informed by the same comparator(s) and studies that integrated the clinical development program of the pharmacological intervention, representing direct links to assess the health outcomes. Indirect evidence represents the opposite. Microsoft Excel® was used to perform descriptive statistics.

RESULTS

Among the 987 relative effectiveness assessment reports analysed, 717 (72.64%) were exclusively supported by direct evidence. Among these, the most used data sources were clinical trials (RCT) (706; 98.47%), followed by the observational studies (OS) + RCT (3, 0.42%), and OS + meta-analysis + RCT (2, 0.28%). A total of 270 (27.36%) relative effectiveness assessments were informed by indirect evidence. Among those, network meta-analysis (110, 41.11%), indirect treatment comparisons (84, 31.11%), and matching adjusted indirect comparisons (MAIC) (31, 11.48%) were the most used data sources.

Table 1 - Data sources used when direct evidence.

Data sources	Nº of reports (%)
RCT	706 (98.47%)
Observational Study and RCT	3 (0.42%)
Observational Study, Meta-analysis and RCT	2 (0.28%)
Review and Observational Study	1 (0.14%)
Bioequivalence Studies	1 (0.14%)
Systematic Review and RCT	1 (0.14%)
Non-RCT	1 (0.14%)
Meta-analysis and RCT	1 (0.14%)
Meta-analysis	1 (0.14%)
Total	717 (100%)

Table 2 - Data sources used when indirect evidence.

Data sources	Nº of reports (%)
Network Meta-analysis and RCT	111 (41.11%)
ITC and RCT	84 (31.11%)
MAIC and RCT	31 (11.48%)
Observational Study and RCT	15 (5.56%)
Meta-analysis and RCT	6 (2.22%)
Network Meta-analysis, ITC and RCT	6 (2.22%)
Network Meta-analysis	3 (1.11%)
ITC	3 (1.11%)
MAIC	3 (1.11%)
NMA, Meta-Analysis and RCT	2 (0.74%)
Systematic Review and RCT	1 (0.37%)
RCT	1 (0.37%)
Observational Study	1 (0.37%)
Observational Study, Meta-analysis and RCT	1(0.37%)
NMA, Meta-analysis and Observational Studies	1 (0.37%)
Systematic review	1 (0.37%)
Total	270 (100%)

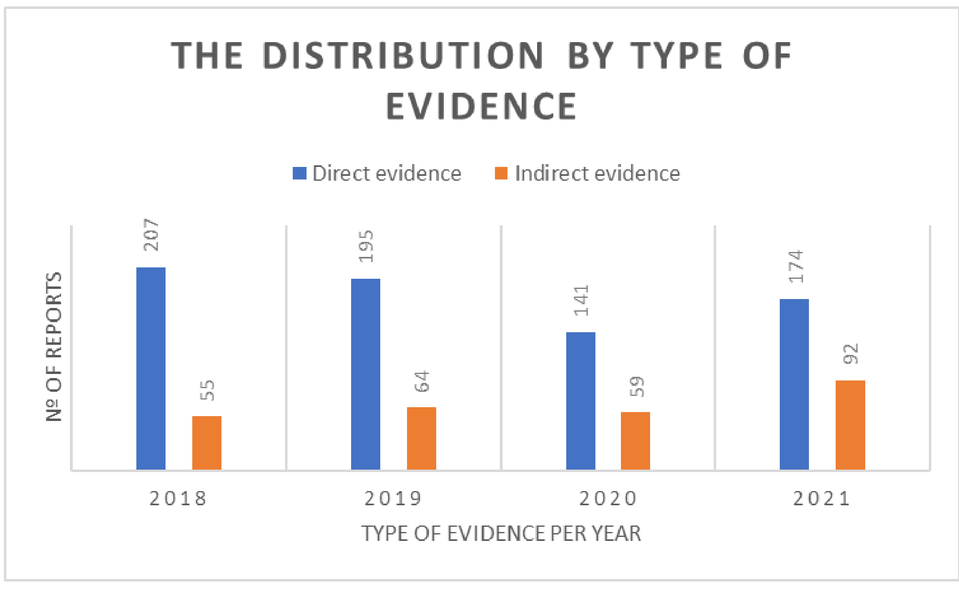

Figure 2 - The distribution by directness of evidence, between 2018-2021.

CONCLUSIONS

Most of the relative effectiveness assessments are informed by direct evidence. Nonetheless, there is a considerable proportion of these procedures that use indirect evidence to identify the therapeutic value of pharmacological interventions, which may denote the need to further improve the design of RCTs for HTA purposes.

REFERENCES

1 - EUNEHTA Comparators & Comparisons. Criteria for the choice of the most appropriate comparator(s) s). Summary of current policies and best practice recommendations. EUnetHTA JA2 . February 2013 (2015)1-23;

2 - Kristensen, F. B., Mäkelä, M., Neikter, S. A., Rehnqvist, N., Håheim, L. L., Mørland, B., ... & Chamova, J. (2009). European network for Health Technology Assessment, EUnetHTA: Planning, development, and implementation of a sustainable European network for Health Technology Assessment. International journal of technology assessment in health care, 25(S2), 107-116.

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