



A Structured Framework for Categorising Unmet Need in HTA: Application to Solid Tumor Assessments

Diana Rebeca Acosta Focil, MD¹, Ahmad Hecham Alani, PharmD¹, Mackenzie Mills, PhD¹, Panos Kanavos, PhD²

¹ Hive Health Optimum Ltd. (HTA-Hive), London, United Kingdom

² The London School of Economics and Political Science (LSE), London, United Kingdom

Background

- Unmet need is frequently considered in health technology assessment (HTA), particularly for oncology medicines, but it remains inconsistently defined and interpreted across agencies, stakeholders, and decision-making contexts.¹
- This inconsistency may limit transparency when unmet need is used to contextualise evidence uncertainty, clinical value, patient relevance, and reimbursement decisions across jurisdictions, highlighting the need for a structured framework to support consistent identification and comparison across HTA reports.²

Objectives

- To develop and apply a structured framework for categorising unmet need statements in solid tumour HTA reports across eight HTA agencies
- To explore whether the presence and type of unmet need are associated with HTA outcome, as a proxy for evidence acceptability.

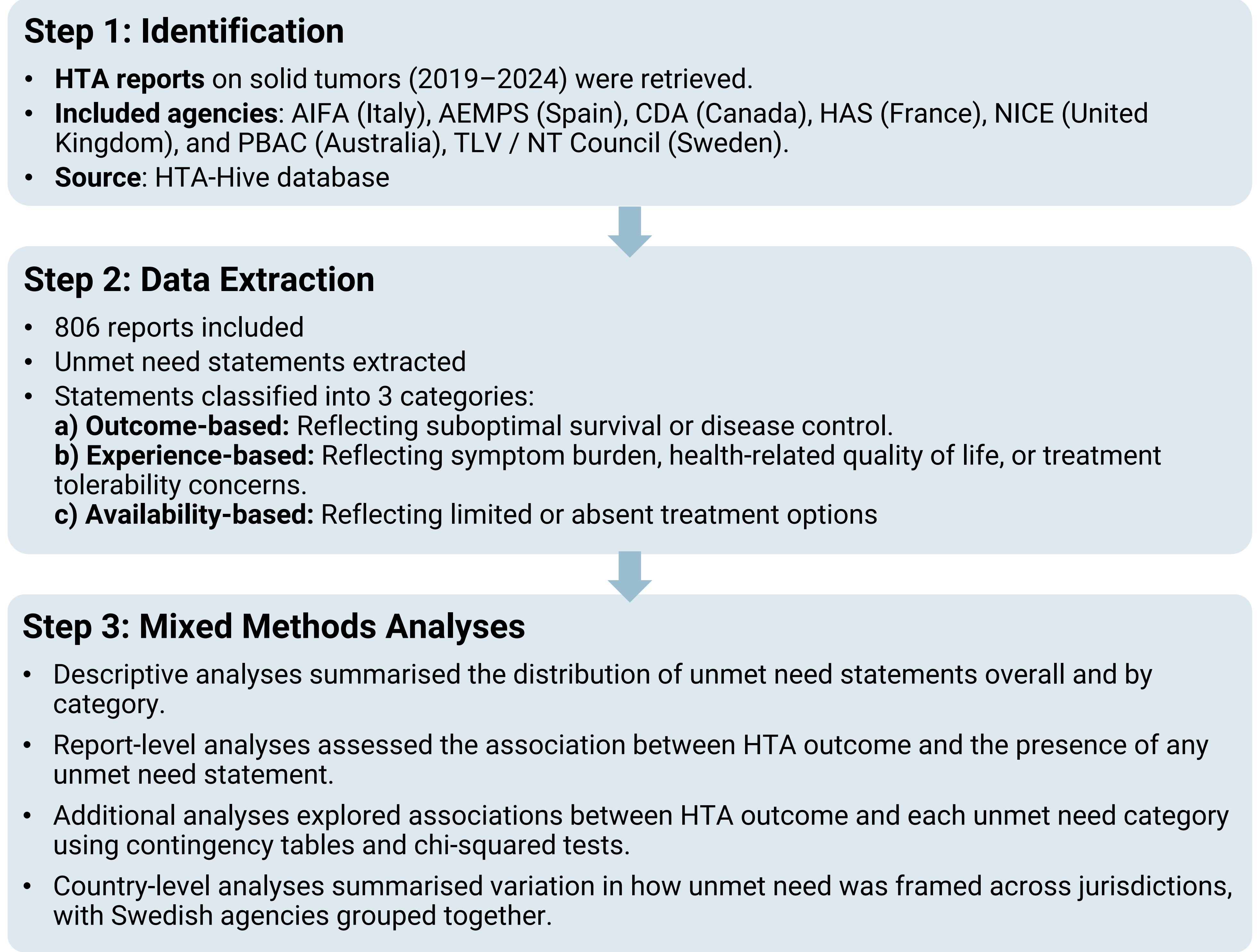
Results

Sample Characteristics		N (%)
Total Sample	HTA reports	806 (100%)
HTA reports by outcome¹	Positive	574 (71.2%)
	Negative	229 (28.4%)
HTA reports by country	Australia	173 (21.5%)
	Canada	111 (13.8%)
	United Kingdom	119 (14.8%)
	Italy	84 (10.4%)
	France	108 (13.4%)
	Spain	125 (15.5%)
	Sweden	86 (10.7%)
	HTA reports by unmet need statement inclusion²	Yes
No	187 (23.2%)	
Total Sample	Unmet need statements ²	688 (100%)
Statements per category	Outcome-Based	298 (43.3%)
	Experience-Based	23 (3.3%)
	Availability-Based	367 (53.3%)

¹ Three reports from Spain without CIPM decisions were excluded.
² Reports may include more than one statement.

- 803 HTA reports** were included in the outcome analysis; **574 (71.5%)** had a positive outcome and **229 (28.5%)** had a negative outcome.
- Most reports included at least one unmet need statement (**618/803; 77.0%**). The presence of any unmet need was **not significantly associated** with HTA outcome ($\chi^2=0.26$; $p=0.61$).
- Availability-Based unmet need was the most frequent category (**53.3%**), followed by Outcome-Based unmet need (**43.3%**) and Experience-Based unmet need (**3.3%**).
- No statistically significant association was observed between HTA outcome and Availability-Based unmet need ($\chi^2=3.81$; $p=0.051$), Experience-Based unmet need ($\chi^2=0.00$; $p=1.00$), or Outcome-Based unmet need ($\chi^2=0.90$; $p=0.34$).

Methods



Abbreviations

AEMPS, Agencia Española de Medicamentos y Productos Sanitarios; AIFA, Agenzia Italiana del Farmaco; HAS, Haute Autorité de Santé (French National Authority for Health); HTA, Health Technology Assessment; NICE, National Institute for Health and Care Excellence; PBAC, Pharmaceutical Benefits Advisory Committee (Australia); TLV, The Swedish Dental and Pharmaceutical Benefits Agency; DTC, Differentiated Thyroid Cancer

Availability-Based Unmet Need

“Patients with advanced or metastatic DTC have few treatment options available in Canada and not all respond to these treatments”
 – CDA

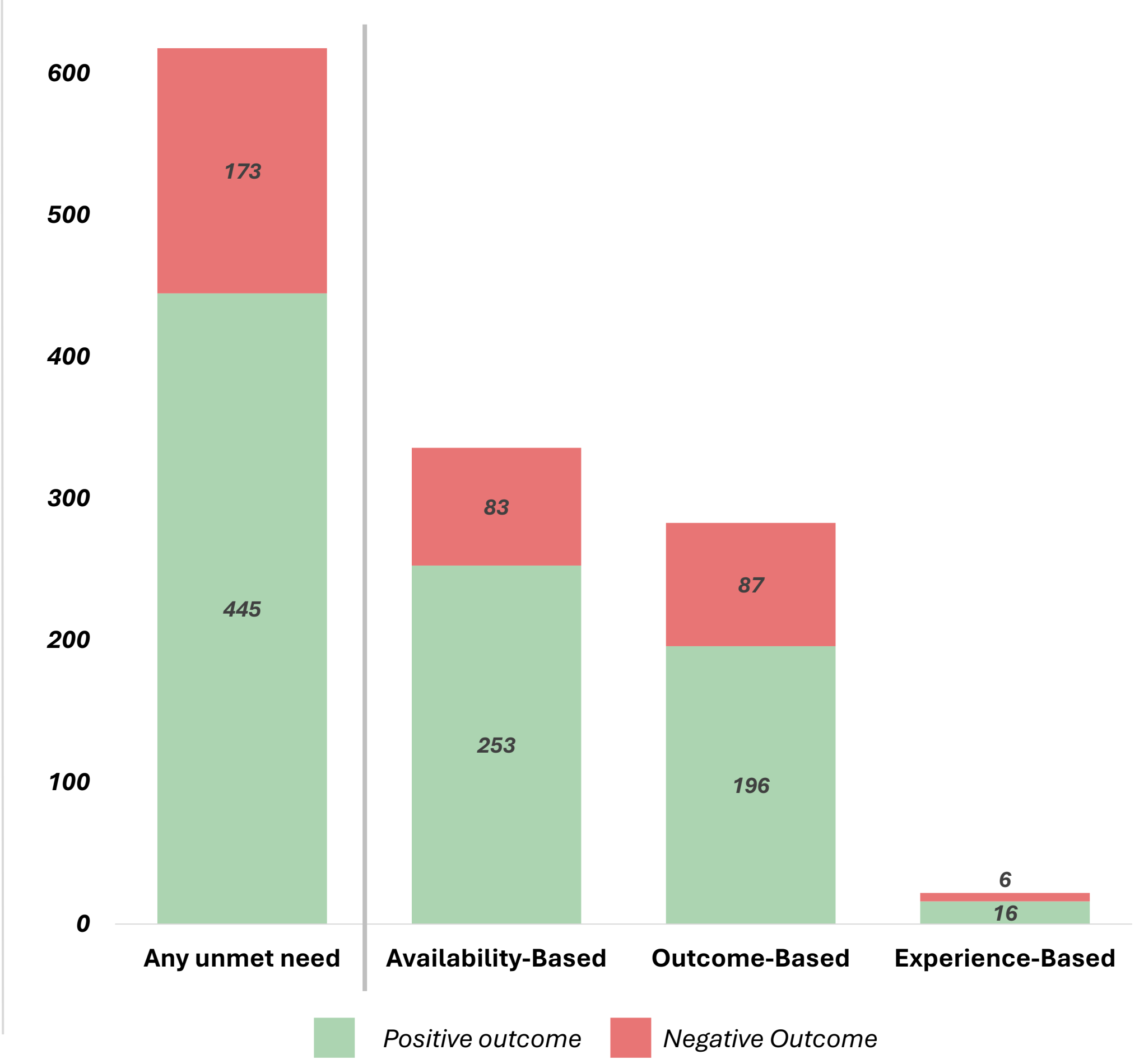
Outcome-Based Unmet Need

“Input from health professionals noted that while metastatic cervical cancers are rare, survival rates for this group are low, and treatment options are limited”
 – PBAC

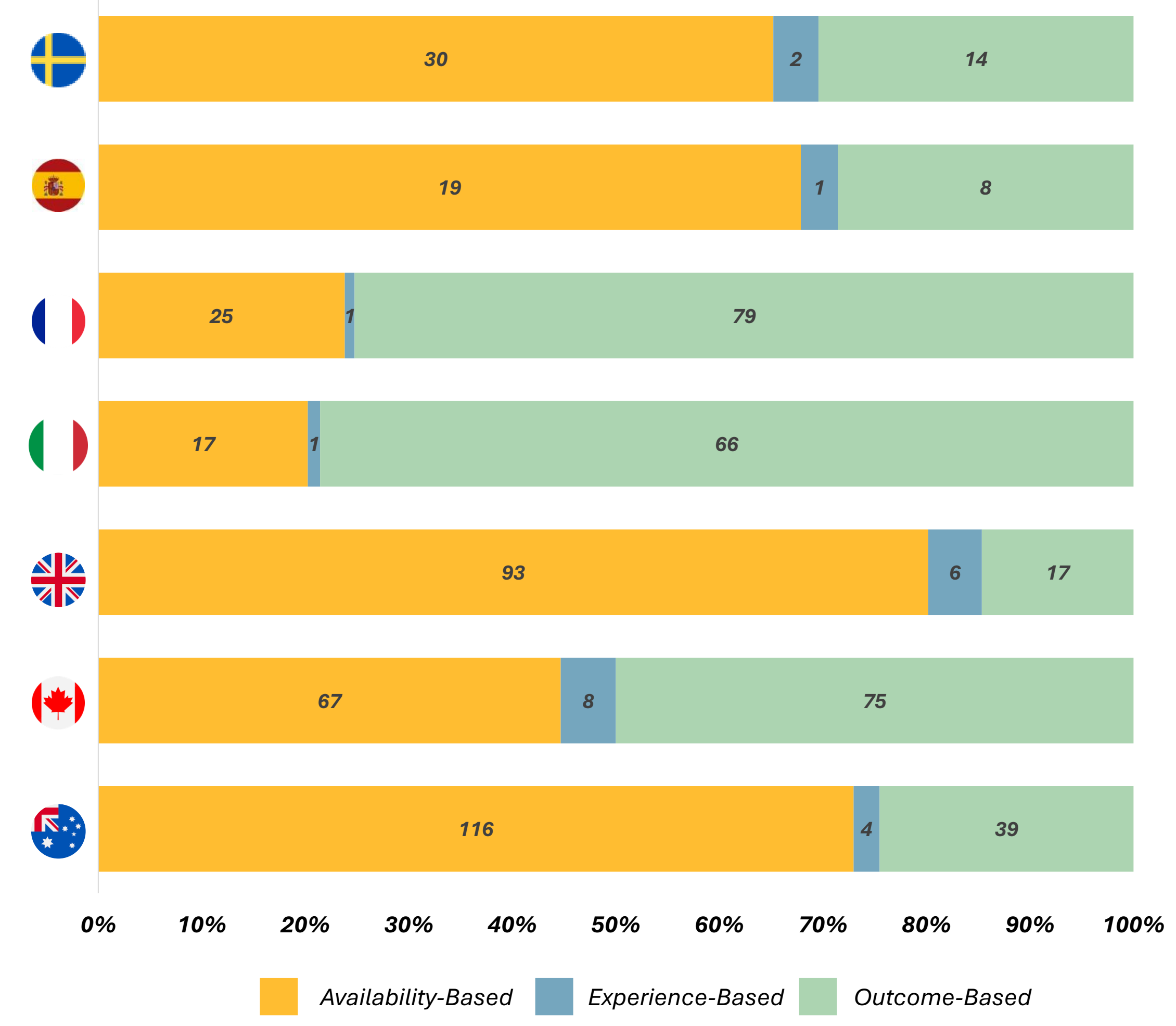
Experience-Based Unmet Need

“Given that the toxicity profile of the nivolumab and ipilimumab combination is not insignificant, there remains a need for safer therapies”
 – AEMPS

HTA outcome by unmet need category



Distribution of unmet need categories by country



Conclusions

- Unmet need was frequently identified in solid tumour HTA reports, with most reports including at least one unmet need statement.
- Availability-Based unmet need was the most common framing, suggesting that agencies most often describe unmet need in terms of limited or absent treatment options.
- No statistically significant association was observed between HTA outcome and the presence or category of unmet need, suggesting that unmet need alone may not consistently predict evidence acceptability.
- The proposed framework provides a structured approach for comparing how unmet need is characterised across HTA agencies and jurisdictions.

References

- Vreman RA, et al. Unmet medical need: an introduction to definitions and stakeholder perceptions. Value Health. 2019.
- Angelis A, Kanavos P. Value-based assessment of new medical technologies: towards a robust methodological framework for the application of multiple criteria decision analysis in HTA. Pharmacoeconomics. 2016.

Contact Information

For further information, please contact d.wells@hiveoptimum.com