



THE UNMET MEDICAL NEEDS CONCEPT – A STRUCTURED ANALYSIS OF ITS APPLICATION IN PUBLIC REGULATORY AND P&R ASSESSMENT DOCUMENTS

Zilke Claessens¹⁺, Isabelle Huys^{1*}, Liese Barbier^{1*}

¹KU Leuven, Department of Pharmaceutical and Pharmacological Sciences, Leuven, Belgium

+Contact: zilke.claessens@kuleuven.be, * shared last authorship



BACKGROUND & OBJECTIVE



Stakeholders, including regulatory and payer decision-makers, appear to have a different understanding of the unmet medical need (UMN) concept where publicly available assessment documents could be a tool for bridging this gap.



Objectives: Publicly available regulatory and pricing & reimbursement (P&R) assessment documents will be studied to

- (i) identify **attributes that are used in public documents to describe the UMN concept**
- (ii) assess the **extent** to which the UMN concept is **discussed**

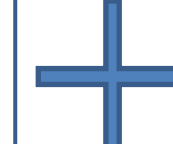
METHODS

1. **Literature review** for the identification of regulatory and market access UMN procedures

2. A **structured document analysis** of (i) European Public Assessment Reports (EPARs) at the central EU regulatory level and (ii) P&R assessment documents at the Belgian national level (CRM documents)

Inclusion criteria for document-selection:

European centralized MA between 2015 and 2020



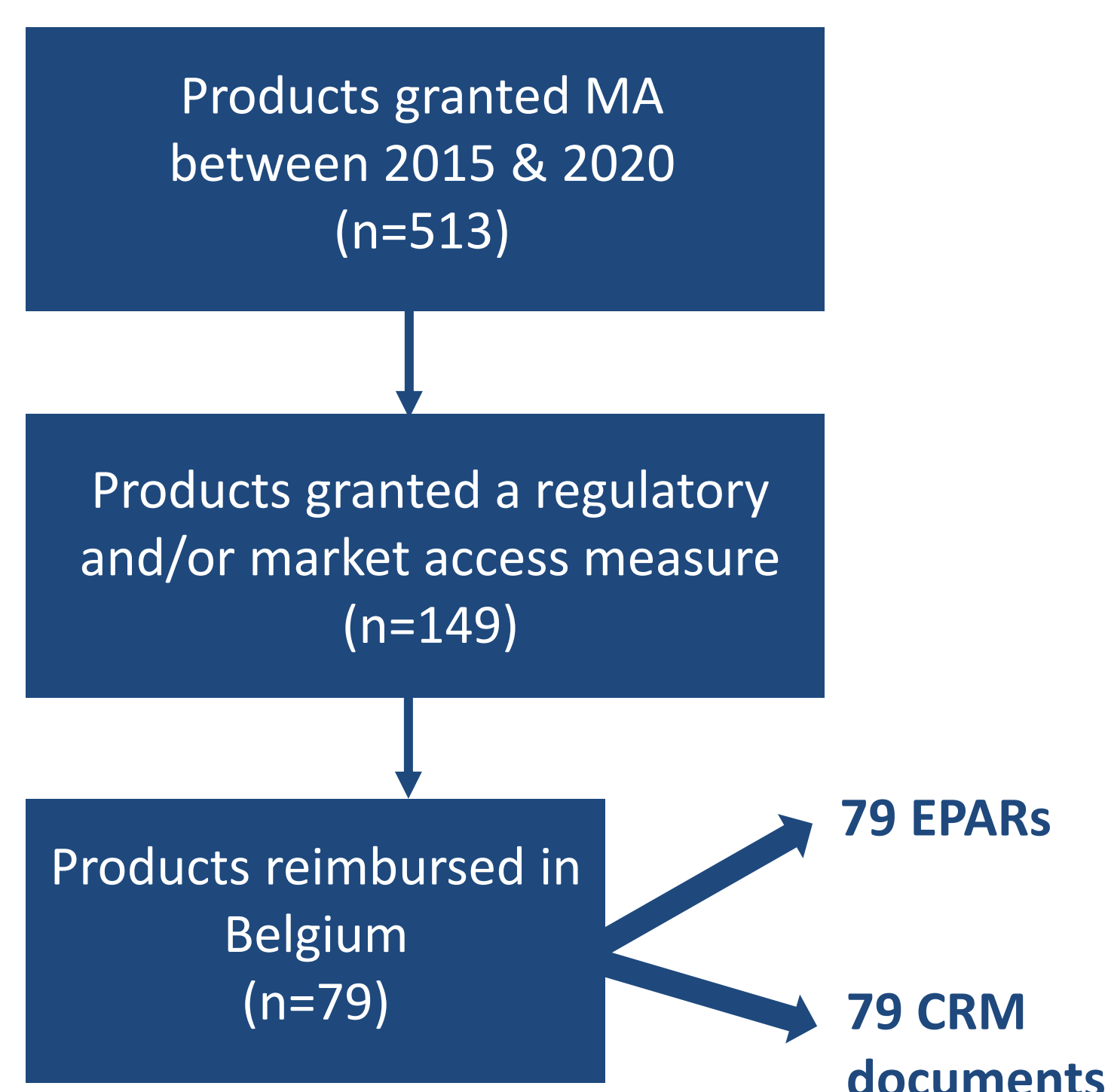
Applied a regulatory or market access UMN procedure



Reimbursed in Belgium

RESULTS

Document inclusion



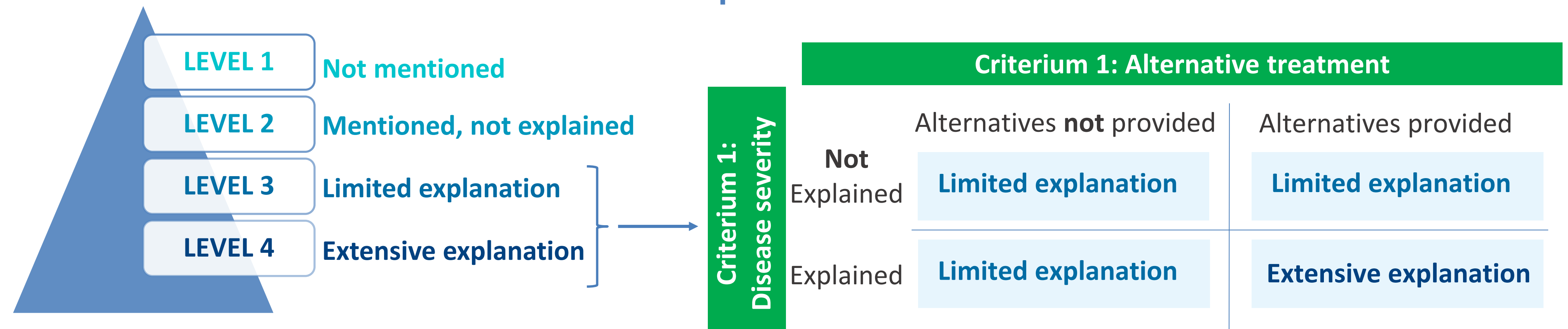
Regulatory UMN procedures

- PRIME scheme
- Orphan designation
- Accelerated approval
- Conditional authorization
- Authorization under exceptional circumstances

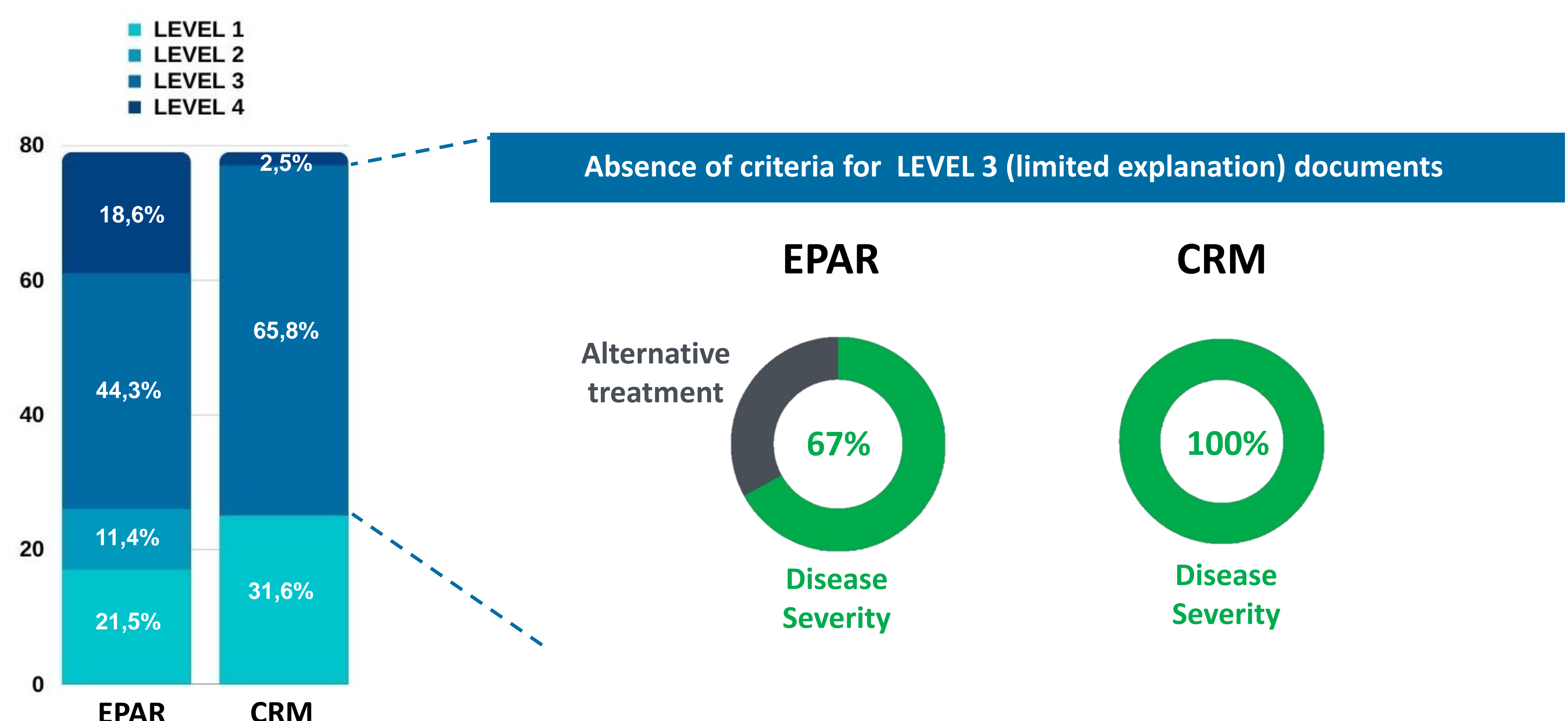
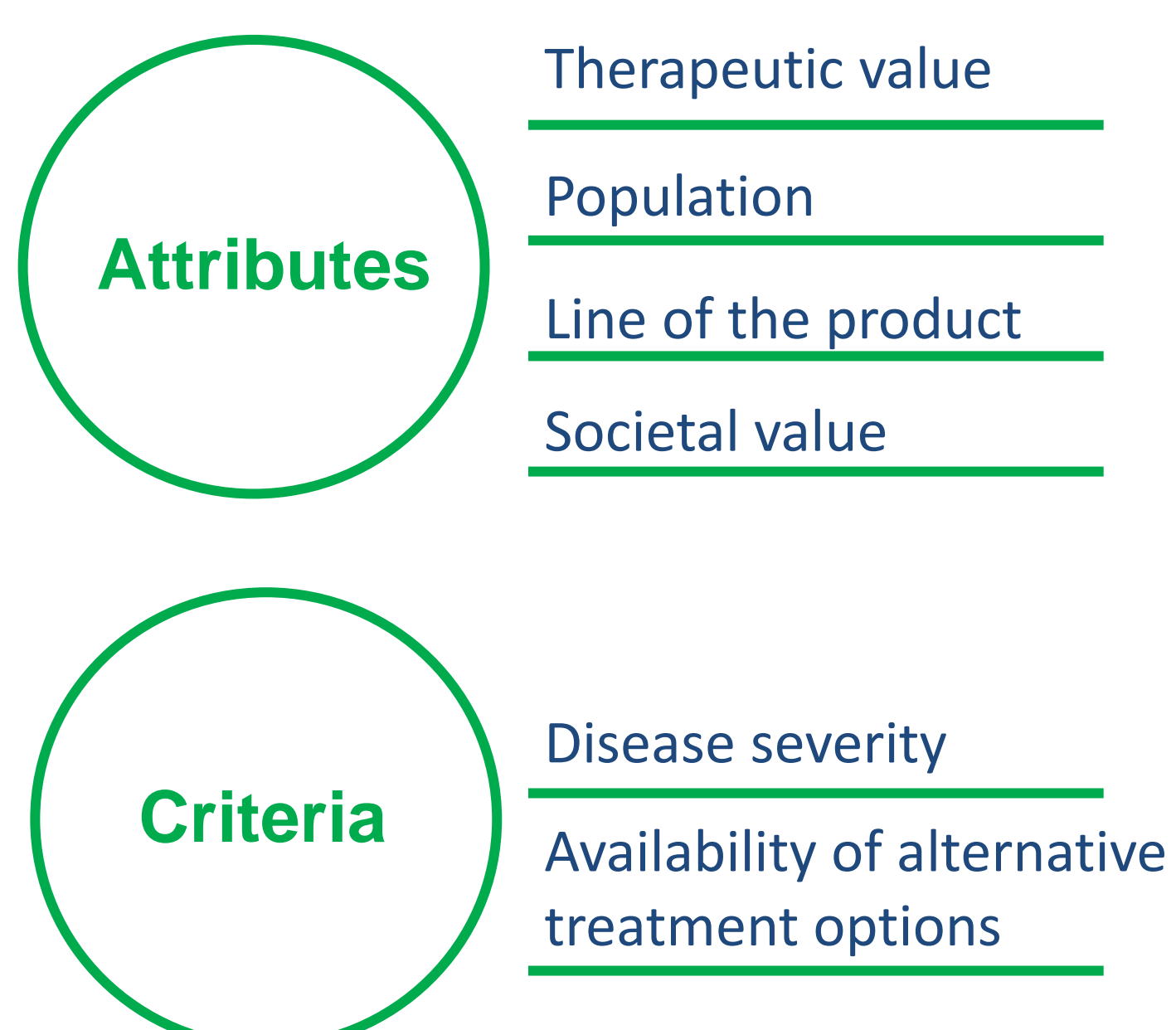
Market access UMN procedures

- Managed entry agreement
- Early access program
- Early temporally reimbursement

The extent of reported UMN information



UMN attribute identification



ABBREVIATIONS

UMN: Unmet Medical Need, P&R: Pricing and Reimbursement, EPAR: European Public Assessment Report, MA: Marketing Authorisation, CRM: Commission Reimbursement Medicines

CONCLUSION

The extent to which the regulatory and P&R assessment documents provide insight into the identification and assessment of UMN in their product evaluation is limited. Only in 18,6% of EPARs and 2,5% of CRM documents the UMN criteria; disease severity, and availability of alternative treatment, were explained. Increased and more systematic reflection on the UMN concept in publicly available documents could:

1

improve transparency in decision making

2

advance structured and systematic application of UMN assessment in decision-making

3

advance the understanding of the UMN concept

ACKNOWLEDGEMENTS

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