





# How is the Unmet Medical Need Concept Applied in Marketing Authorisation and P&R Assessments? A Structured Document Analysis

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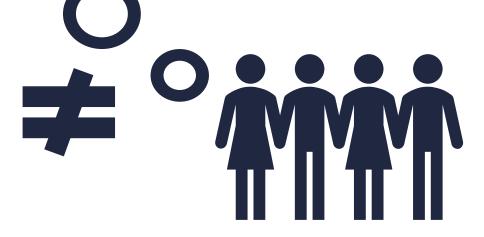
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### **BACKGROUND AND OBJECTIVES**



documents, and

criterion for specific regulatory and market access procedures aimed at expediting access to highly necessary medicines



varying interpretations of the UMN concept among stakeholders

#### **Objectives:**



(B) identify the **elements** used to substantiate the UMN in specific cases.

the application of the UMN concept in assessment

#### **METHODS**

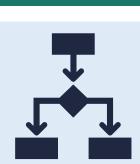


- 1. Identification of all medicines for which a supportive measure was applied at European and Belgian level between 2015-2020
- **Priority Medicines**
- Orphan Medicinal Product Designation
- **Condition Marketing Authorisation**
- **Authorisation Under Exceptional** Circumstances
- **Accelerated Assessment**



- Compassionate Use Program
- Medical Need Program
- Managed Entry Agreement





2. Development of extraction framework



3. Systematic screening of assessment (EPAR & CRM) documents and data extraction



4. Descriptive analysis & qualitative thematic framework analysis

#### INCLUDED DOCUMENTS

Number of products included in Belgian CUP/MNP N = 207

Number of products included in Belgian MEA N = 372

Number of products with EMA measure, authorised between 2015-2020 N = 111

Authorised between N = 97

CRM doc. 2015-2020 Duplicates N = 103N = 66included N = 142

EPAR doc. N = 142

### **UMN CRITERIA**

Impact on individual Impact on society

Impact on QoL

Physical well-being

Psychological well-being

Autonomy

Social life

Impact on mortality

Life expectancy

#### Legend: EPAR + CRM **EPAR**

CRM

## Disease severity and burden

Impact on organisation of

healthcare system Frequent hospitalizations

Burden for healthcare systems

More informal care needed

More access to care is needed

**Economic impact** 

Impact on health of the population

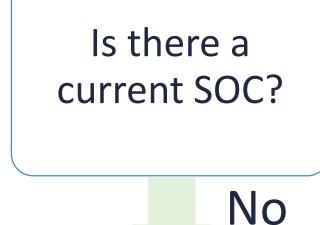
Global health emergency

Impact on population health

Threat to public health

No

**Availability of alternatives:** 



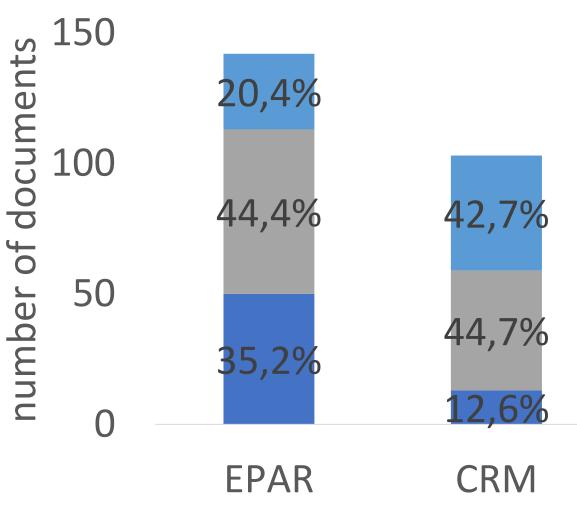
Is the Yes alternative modifying/ curative? No Yes Is the alternative reimbursed?

Yes Is there still room for improvement

Yes

Possible existence of **UMN** 

# FREQUENCY AND CONSISTENCY OF APPLICATION OF THE UMN CONCEPT



Mentioned not explained

Mentioned and explained

Not mentioned

- **Intra-document Discrepancies:** Within the same assessment document, one section acknowledged the UMN, while others did not.
- EMA vs. EPAR: Some medicines were considered UMN-eligible by EMA but denied in EPAR documents.
- **EPAR vs. CRM doc.:** Medicines recognized as addressing UMN at the European level were not acknowledged at the national level in CRM documents.

#### CONCLUSION

**Need for Transparency**: Increased, systematic detailing of UMN rationale in public documents can improve transparency.



Stakeholder Understanding: Clearer documentation could support better stakeholder comprehension of UMN concepts and decisions.

#### **ACKNOWLEDGEMENTS**

**Limited Documentation**: Current

assessment documents only partially

address UMN criteria.

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#### LIST OF ABBREVIATONS

CRM: Commission Reimbursement Medicine, doc.: document, EMA: European Medicines Agency, EPAR: European Public Assessment Report, UMN: unmet medical need, QoL: Quality of Life, SOC: Standard of care.