

Multi-stakeholder perspectives on interpretation, challenges, and ways forward for the unmet medical need concept: semi-structured interviews

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BACKGROUND AND OBJECTIVES

Policy evolutions:

Pharmaceutical strategy for Europe, Nov. 2020

- Current incentive models do not provide a sustainable solution
- UMN as a cornerstone in R&D prioritisation
- Call for more alignment between the stakeholders (e.g. patients, HCP's, regulators)

Proposal Pharmaceutical Regulation, Mar. 2023

- Adjusted UMN definition
- Distinction between UMN and high UMN in the context of rare diseases
- Link between UMN and longer market exclusivity and other regulatory actions

Problem statement:

Disagreement among stakeholders on the **definition** of UMN and its criteria

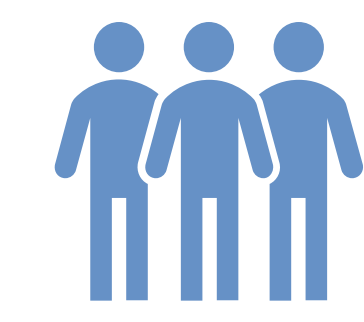
Challenges with the **practical embedding** of UMN in decision-making

Objectives:

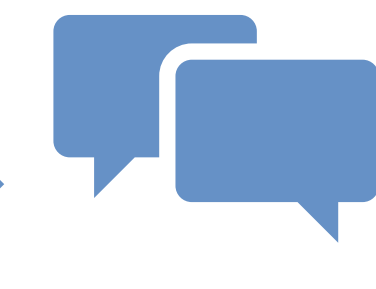
Identify stakeholder perceptions towards & interpretation of UMN, differences, and common grounds

Provide recommendations on the embedding of UMN in decision-making to support R&D in those areas with the highest needs

METHODS AND DESCRIPTIVES



Purposive sampling



Semi-structured interviews



Ad verbatim transcription



Thematic framework analysis

| N total =45 | Pharma industry & researchers (N=13) | Policymakers, regulators, HTA bodies, payers (N=12) | HCPs (N=9) | Patient (-representatives) (N=11) |
|-------------|--------------------------------------|---|------------|-----------------------------------|
| EU | 8 | 7 | 1 | 5 |
| National | 5 | 5 | 8 | 6 |

Expertise in e.g. Oncology, Rare diseases, Paediatrics, Respiratory diseases, and Metabolic diseases

DO WE NEED THE CONCEPT OF UMN?

PRO

- Contribute to **transparency and predictability** of decision-making
- Allow for more **alignment** between the different stakeholders
- Tool to **allocate resources** to the areas that need it the most
- **Stimulate research** in areas that are unaddressed

CONTRA

- If the concept is vague, it is **open for interpretation** and will not be workable
- If the concept is **concrete**, there is a risk of **excluding diseases** and negatively **impacting access** to some patients
- Alternative: **priority areas**

Industry and policy stakeholders explained the importance of an open and flexible definition:

- Needs change over time → allow for **dynamic** nature
- Allow for a **case-by-case assessment**
- Allow for **adjustment to different application settings**

DEFINING UMN

1 Bringing the definition into practice

Complementary to a revised definition, stakeholders over the different groups were in favour of an **UMN identification framework** for the translation into practice

- Including UMN criteria (e.g. mortality, quality of life, severity, time of lacking treatment, urgency to a treatment)
- Robust methodology (e.g. survey, patient interviews, patient involvement)
- Adjustable to the decision-making context

2 Reducing focus on “medical” or pharmaceutical development

Some stakeholders emphasize that healthcare is **broader than only medication**: e.g. research needs, clinical care needs, psycho-social needs

➔ Broaden the definition

3 Distinction between patient and societal needs

- Several stakeholders suggest to make a distinction between patient needs and societal needs
- One stakeholder explains that UMN is a part of this broader understanding

APPLICATION OPPORTUNITIES THROUGHOUT THE DRUG LIFE CYCLE

Research and development

1. **Resource allocation** of investments by the pharmaceutical industry and research investment funds

Marketing Authorisation

1. As a requirement for the inclusion in EMA regulatory **expediting actions** (e.g. PRIME, Orphan Designation, Conditional Marketing Authorisation)
2. Criterium for longer **market exclusivity**

Market Access

1. As a requirement for inclusion in **early access schemes** i.e. Medical Need List – INAMI
2. As a requirement for the applicant to obtain **early temporary reimbursement**

Clinical practice

1. In the development of **clinical guidelines**

List of abbreviations

UMN: unmet medical need, HCP, health care professional, HTA: health technology assessment, EMA: European Medicines Agency, INAMI: National Institute for Health and Disability Insurance in Belgium

Acknowledgements

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CONCLUSION

1. **Open, flexible definition** of UMN, embedded in law
2. Translation to an **UMN identification framework** for the application in practice
3. The framework must be **applicable throughout the whole drug life cycle**



Fundamentals for a **needs-driven health care framework** that directs research and development efforts towards the highest needs