

# Towards more Unmet Need-Driven Product Development and Healthcare Decision-Making

Stakeholder views on needs, challenges,  
and proposed approaches

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# Unmet Medical Need: EU policy development

## 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 for Revision of Pharmaceutical Legislation April 2023



- Adjusted UMN definition
- Distinction between UMN and high UMN in the context of rare diseases
- Link between UMN and regulatory protection period and EMA regulatory support

# Unmet Medical Need: Open Questions

- **Agreement** on the **importance** of focusing research and development on unmet needs but
- **Varying interpretations and views** towards **meaning, and definition and its criteria** of unmet needs; broad versus narrow definition
- **How to practically embed** in product evaluation and healthcare decision-making?
- **Methods and frameworks for identification?**  
Appropriate for different disease contexts? Possible to create generic tools? Risks? Case-by-case assessment?
- **Stakeholder views?**



4. A reform boosting innovation and EU competitiveness

An effective **incentives framework** for innovation, access and **addressing unmet medical needs**

Let's discuss the future  
of Unmet Medical Needs  
(UMN) in EU Policies

Report from a multi-stakeholder workshop



EPF proposal for a patient-centred framework  
for defining Unmet Medical Needs

October 2023

# EU Pharmaceutical Strategy for 2020



## Delivering for patients



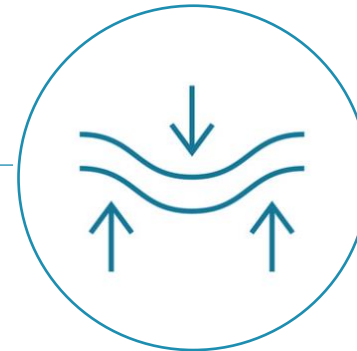
- Medicine access & affordability
- **Unmet medical need**

## Fair competition & innovation



- Technology and digitalisation
- Flexible regulatory framework

## Enhancing resilience



- Supply chain management
- Environmental sustainability

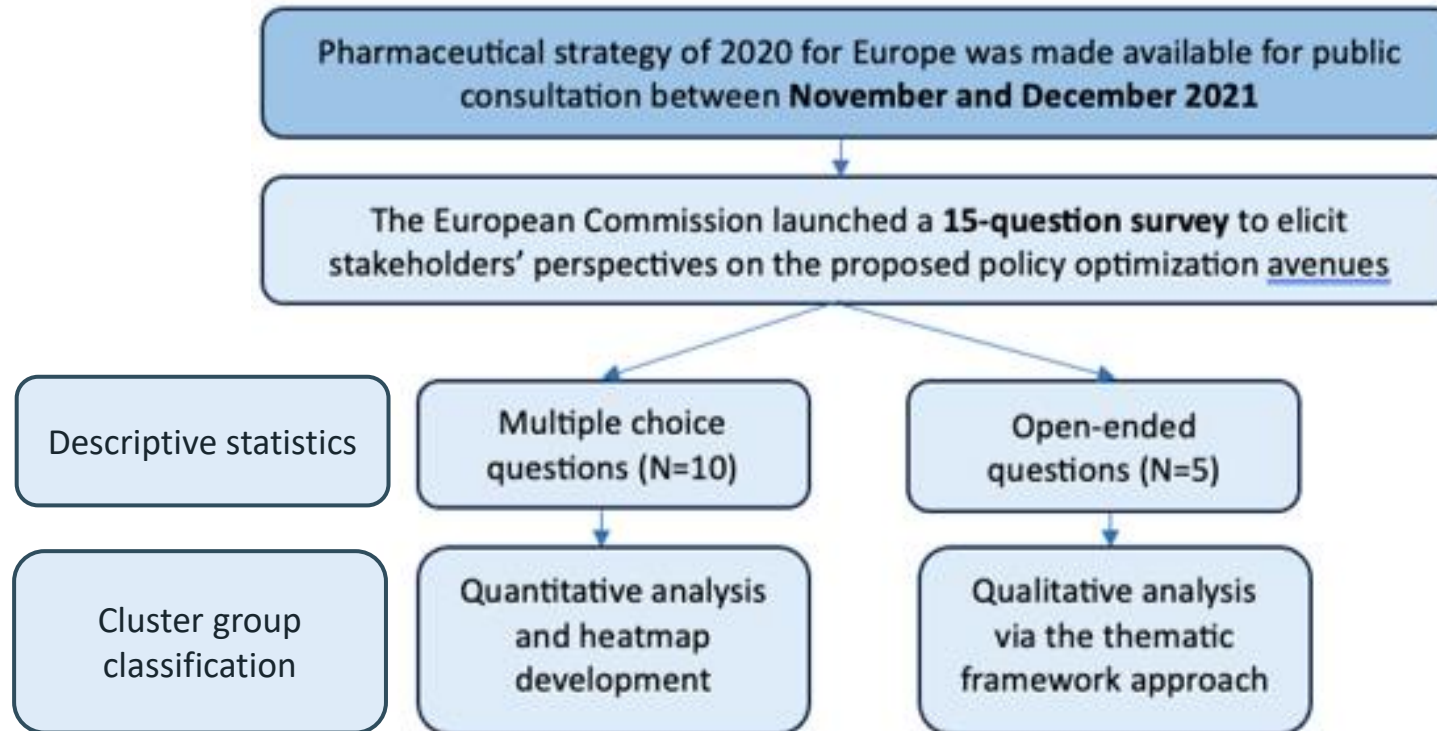
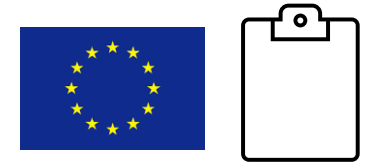
## Ensuring a strong EU voice in the world



- Stakeholder cooperation
- Member state coordination

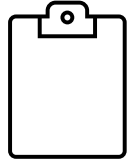
# EU Pharmaceutical Strategy Survey

## Analysis of responses

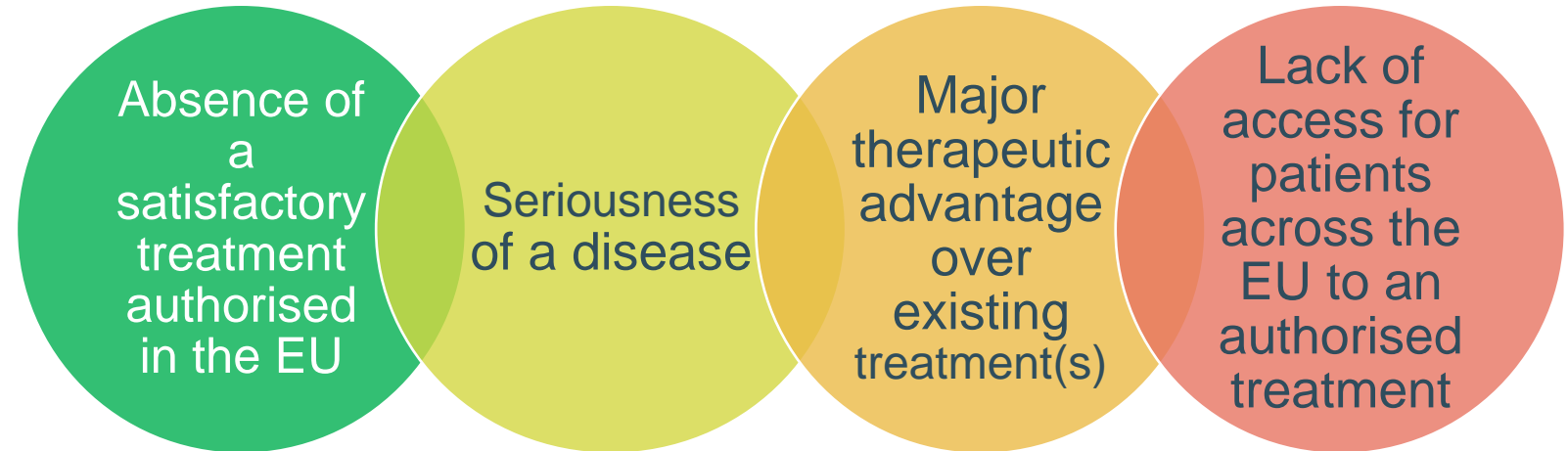


Stakeholder group	N (%)
Developers	172 (36%)
End-users	90 (19%)
HCP	82 (17%)
Public body	36 (7,5%)
Research	33 (7%)
Other	66 (13,5%)
<b>Total</b>	<b>478</b>

# Defining UMN - Disease severity & absence of authorised treatment important criteria to include



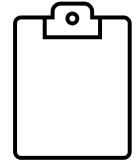
**Developers**



**Other stakeholder groups**



# Defining UMN - Disease severity & absence of authorised treatment important criteria to include



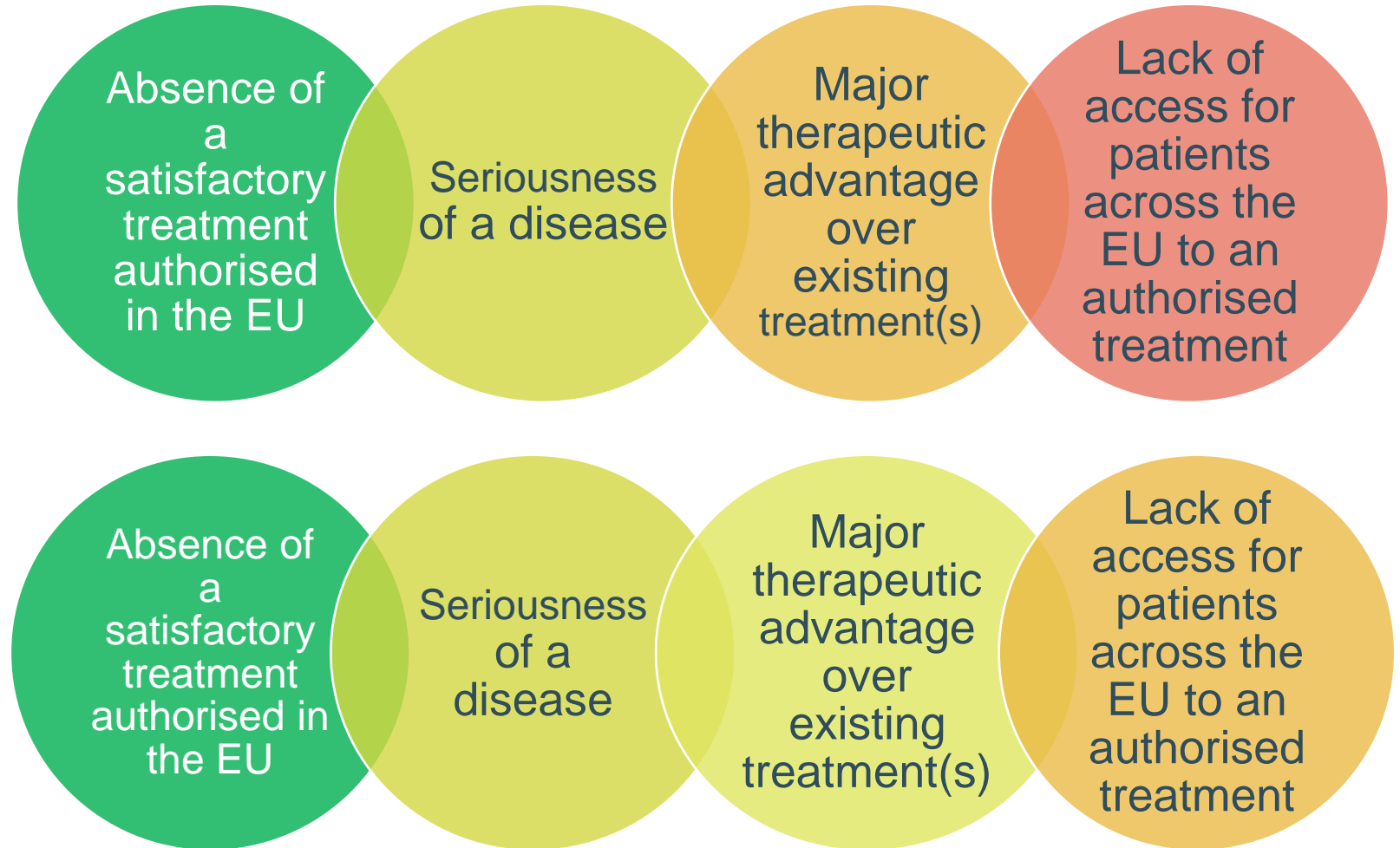
Meet one or multiple criteria to be considered an UMN?

Scale rather than binary decision?

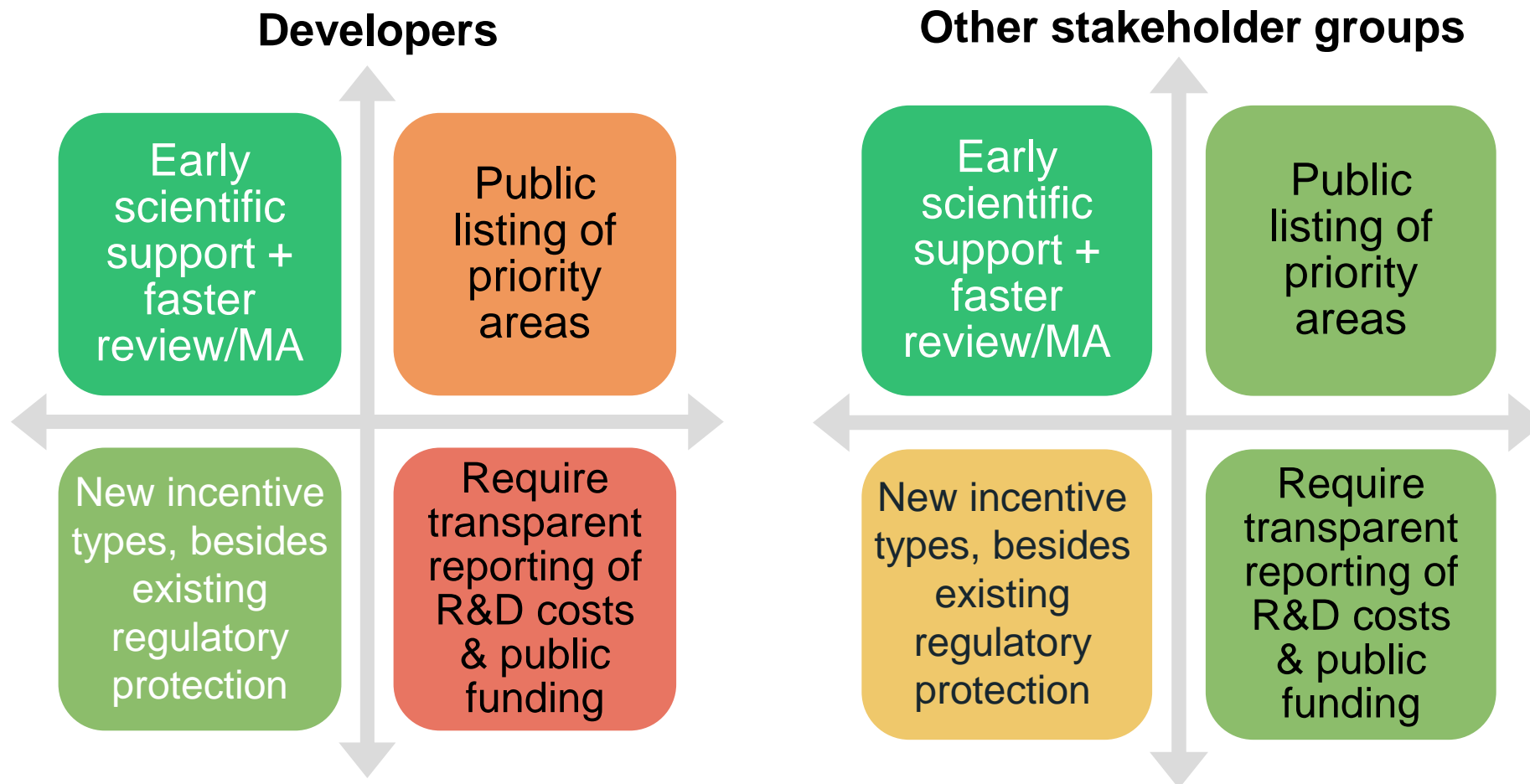
Criteria should be made quantifiable

Definition should be updated over time

Patient satisfaction with current therapy?



# Proposals to support innovation, also in UMN areas – Varying stakeholder views



1-2	Not important
2-3	Fairly important
3-4	Important
4-5	Very important

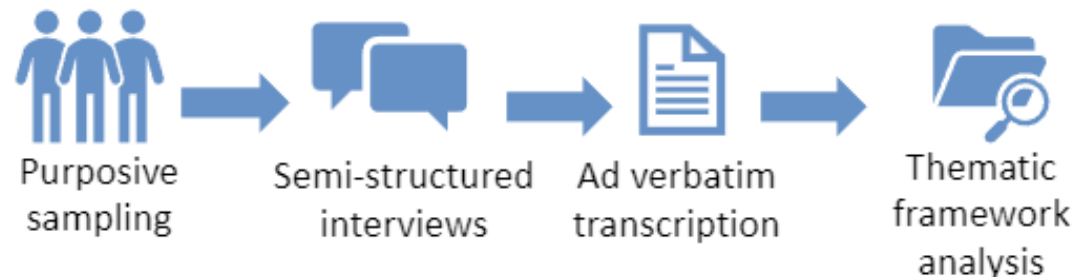


# Stakeholder views on UMN concept

## Qualitative research study



- **Identify stakeholder perceptions** towards UMN, differences & commonalities
- **Provide recommendations** on the identification, and application of UMN in decision-making to support R&D in those areas with the highest needs
- **Semi-structured interviews with expert stakeholders**, incl. developers, policymakers, regulators, HTA representatives, payers, HCPs, and patient representatives, across Europe (2023)



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

# Stakeholder views on UMN definition



UMN definition?

Application: How to bring the definition into practice?

Scope: Only medicinal products?  
Patient vs Societal need?



- ↑ **Transparency and predictability** of decision-making
- ↑ **Alignment** between different stakeholders
- ↑ **Allocation of resources** to areas that need it most
- ↑ **Research** in areas that are unaddressed

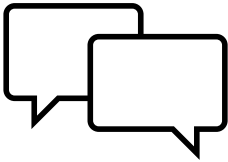


**Broadness/narrowness of definition:** open for interpretation, practical difficulties in application vs risk of excluding diseases and negatively impacting access to some patients



**Broad definition** with more **concrete translation** in the different **application settings, e.g. under the form of a guiding framework** with appropriate **criteria** and **case-by-case assessment**

# Stakeholder views on application



UMN definition?

Application: How to bring the definition into practice?

Scope: Only medicinal products?  
Patient vs Societal need?



- Stakeholders in favour of an **UMN identification framework** consisting of
- **UMN criteria** (e.g. mortality, quality of life, severity of disease, urgency to a treatment, impact of condition on life expectancy, appropriateness of current standard of care)
  - **Robust methodology** (e.g. survey, patient interviews, patient involvement)
  - **Adjustable** to the **decision-making context** and **disease context**

# Stakeholder views on scope



UMN definition?

Application: How to bring the definition into practice?

Scope: Only medicinal products?  
Patient vs Societal need?



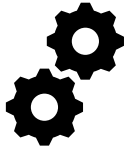
Some stakeholders emphasize need for a broader focus **than only medicinal products** intended to treat; e.g. clinical care needs, psycho-social needs

- Several stakeholders suggest to make a distinction between **patient (therapeutic) needs** and **societal needs**
- UMN as part of the broader patient need

# Concluding remarks



Definition of UMN should be **sufficiently broad**, and go hand-in-hand with a well **thought out application framework**, allowing **case-by-case assessment** and tailoring to the respective drug life cycle phase



Development and piloting of **UMN identification frameworks** for application in practice is important; ongoing efforts, e.g. KCE NEED project



**Meaningful patient involvement throughout** the development of definition and framework, as well as involvement in the application process **is crucial**

## Acknowledgements

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# THANK YOU

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