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

- 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)

- 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

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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?

EU Pharmaceutical Strategy for 2020

Delivering for patients
- Medine 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

Pharmaceutical strategy of 2020 for Europe was made available for public consultation between **November and December 2021**

The European Commission launched a **15-question survey** to elicit stakeholders’ perspectives on the proposed policy optimization avenues.

### Stakeholder group

<table>
<thead>
<tr>
<th>Stakeholder group</th>
<th>N (%)</th>
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<tbody>
<tr>
<td>Developers</td>
<td>172 (36%)</td>
</tr>
<tr>
<td>End-users</td>
<td>90 (19%)</td>
</tr>
<tr>
<td>HCP</td>
<td>82 (17%)</td>
</tr>
<tr>
<td>Public body</td>
<td>36 (7.5%)</td>
</tr>
<tr>
<td>Research</td>
<td>33 (7%)</td>
</tr>
<tr>
<td>Other</td>
<td>66 (13.5%)</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>478</strong></td>
</tr>
</tbody>
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Defining UMN - Disease severity & absence of authorised treatment important criteria to include

Developers
- Absence of a satisfactory treatment authorised in the EU
- Seriousness of a disease
- Major therapeutic advantage over existing treatment(s)
- Lack of access for patients across the EU to an authorised treatment

Other stakeholder groups
- Absence of a satisfactory treatment authorised in the EU
- Seriousness of a disease
- Major therapeutic advantage over existing treatment(s)
- Lack of access for patients across the EU to an authorised treatment

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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?

Absence of a satisfactory treatment authorised in the EU

Seriousness of a disease

Major therapeutic advantage over existing treatment(s)

Lack of access for patients across the EU to an authorised treatment

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

Developers

- Early scientific support + faster review/MA
- Public listing of priority areas
- Require transparent reporting of R&D costs & public funding
- New incentive types, besides existing regulatory protection

Other stakeholder groups

- Early scientific support + faster review/MA
- Public listing of priority areas
- New incentive types, besides existing regulatory protection
- Require transparent reporting of R&D costs & public funding

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

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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)

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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**

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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 a **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

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

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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.
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THANK YOU

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