EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Unmet medical need: an introduction to definitions and stakeholder perceptions

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Problem statement

Need to find a balance between the encouragement of **early patient access** and promoting increased **certainty** considering the benefits and harms

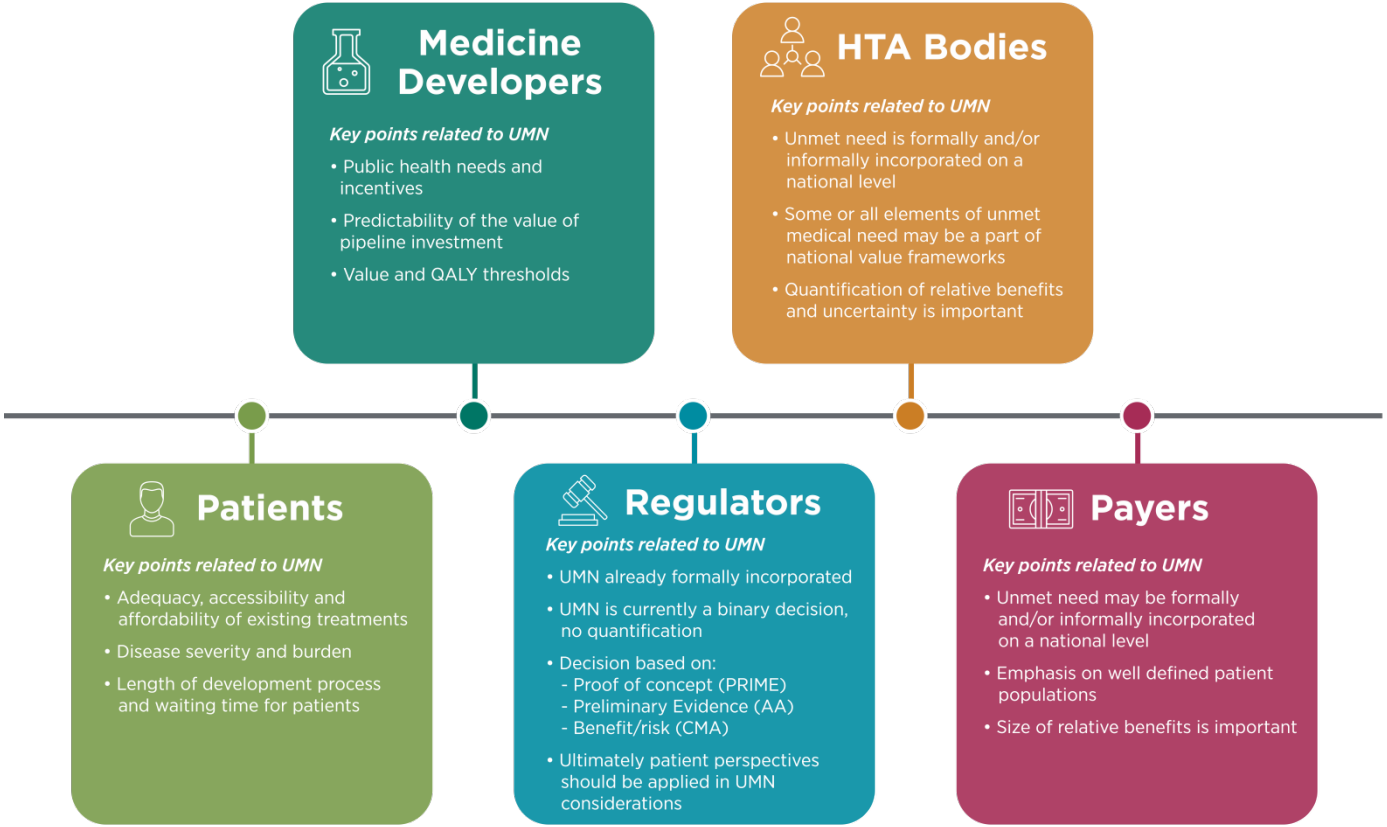
Challenge from **regulatory and development** considerations to decision making on **reimbursement and pricing** of new health technologies

Stakeholders in different countries struggle to find an appropriate relation between **UMN and reimbursement** levels, leading to inter-country differences

A combined exercise

- A scoping and a grey literature review to identify definitions of UMN in literature and on stakeholder websites
- Definitions categorized and discussed in semi-structured group discussions* and open session workshops with a broader audience

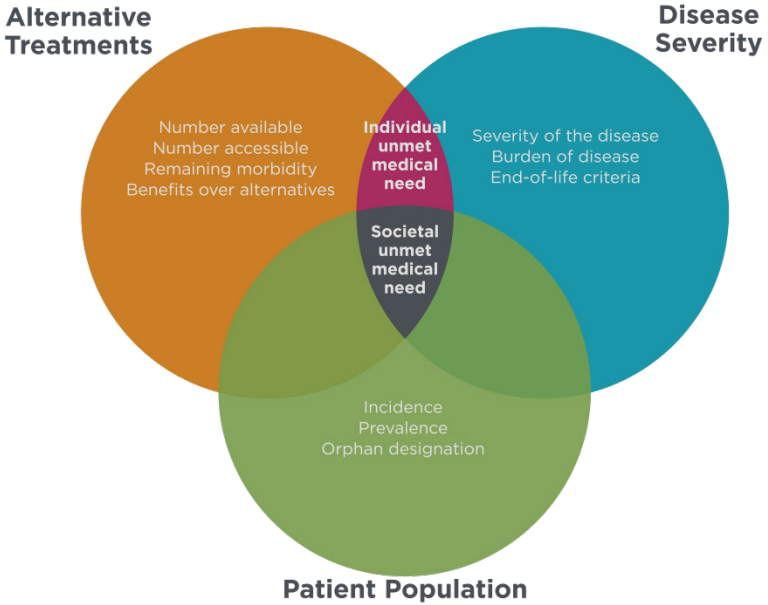
*groups composed of patients reps, HTA, payers, industry and regulators


Medicine Developers
Key points related to UMN
• Public health needs and incentives
• Predictability of the value of pipeline investment
• Value and QALY thresholds
HTA Bodies
Key points related to UMN
• Unmet need is formally and/or informally incorporated on a national level
• Some or all elements of unmet medical need may be a part of national value frameworks
• Quantification of relative benefits and uncertainty is important
Patients
Key points related to UMN
• Adequacy, accessibility and affordability of existing treatments
• Disease severity and burden
• Length of development process and waiting time for patients
Regulators
Key points related to UMN
• UMN already formally incorporated
• UMN is currently a binary decision, no quantification
• Decision based on:
- Proof of concept (PRIME)
- Preliminary Evidence (AA)
- Benefit/risk (CMA)
• Ultimately patient perspectives should be applied in UMN considerations
Payers
Key points related to UMN
• Unmet need may be formally and/or informally incorporated on a national level
• Emphasis on well defined patient populations
• Size of relative benefits is important

Results

- The grey literature review delivered 16 definitions of UMN out of 34 online sources consulted.
- Sources mention UMN as relevant to patients or institutional processes or decisions, yet failed to define how it is established
- Definitions consider alternative treatments, disease severity and patient population

Elements included in definitions



Conclusions

1. UMN definitions should include available treatments as well as some form of disease severity or burden.

- population size may only be relevant for specific scopes.

2. No alignment on how to measure each of these three elements.

3. Agreement that when measuring the size of the patient population, UMN would increase with growing population size if a societal/population perspective was considered

Conclusions

3. Noted perception that very small patient groups (for example those defined by orphan designation) have a relatively higher UMN.

→ contrary to the intuition that societal unmet medical need grows with larger populations (related to low number of available treatments for such small populations)

4. Measurement of UMN may be different when viewed from an individual patient versus population perspective

Any questions?

Further information

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