

Session: Medicines for every disease – return on investment vs unmet clinical needs.

Unmet Medical Needs. Conceptual and ethical issues

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Disclosure of conflict of interests

No conflict of interests to disclose



3 Questions

- The concept of „Unmet Medical Need“ has been defined heterogenously
 - True/false?
- To determine medical needs encompasses value judgments
 - True/false?
- To declare and prioritize a „healthcare need“ requires additional criteria and a weighing of these criteria
 - True/false?



Outline

- „Unmet Medical Need“ (UMN) – what do we mean?
- (Unmet) Medical Need – Conceptual and normative issues
- Prioritizing funding of treatment and research. Substantiative and procedural criteria



Unmet Medical Need – What do we mean?

Example 1:

Lack of effective treatment for patients with Alzheimer Dementia

Example 2:

A new targeted treatment for a small molecularly defined subgroup of patients with cancer for which there exists established „one size fits all treatment“



Unmet medical Need – Definitional elements

“... 'unmet medical needs' means a condition for which there exists **no satisfactory** method of diagnosis, prevention or treatment **or**, even if such a method exists, in relation to which the medicinal product concerned will be of **major therapeutic advantage** to those affected”

European Commission 2006



Unmet Medical Need – Definitional elements

- (1) definitions that only include availability of alternative treatments;
- (2) definitions that include availability of alternative treatments and **some form of disease severity or disease burden**;
- (3) definitions that include availability of alternative treatments, some form of disease severity or disease burden, **and size of the population**.

Vreman RA et al. 2019



Medical Need

Impairment of normal species functioning restricts an individual's opportunity to pursue life plans.

Accordingly “the social obligation to meet health-care needs derives from the more general social obligation to guarantee fair equality of opportunity.”

Daniels 1985, p. 39 – quoted by Marckmann 2004



(Unmet) Medical Need – Conceptual and normative aspects

Person X having a health-care need means that X can be benefited by a healthcare intervention Y to move X from the current level of the health-care value Z to a higher level of Z



(Unmet) Medical Need – Conceptual and normative aspects

1. Severity of the disease

- Length/ Quality of life
- Individual / population perspective
- Indicator for “size” healthcare need (gap between actual state and reference state)
- “Disease–” versus “condition–severity”

Sandmann and Hofmann 2019



(Unmet) Medical Need – Conceptual and normative aspects

2. Treatment options

- No treatment option
- Treatment available
 - Impact in relation to acceptable/optimal state of health
 - Evidence for impact
- Access to treatment



(Unmet) Medical Need – Conceptual and normative aspects

3. Rare diseases

- Challenges for development of treatment for rare diseases
- Rare diseases may be severe (or not)
- Critical reflection on adequacy of incentives for rare diseases in light of “orphanisation”



(Unmet) Medical Need – Conceptual and normative aspects

Determining (Unmet) Medical Need – Substantiative Criteria

1. Professional / technical enterprise
i.e. to determine whether new treatment can reach certain outcome, to determine which kind of evidence is needed etc

2. Normative enterprise
 2. i.e. whether there is a need and corresponding moral obligation to provide (additional) healthcare

Marckmann 2004



(Unmet) Medical Need – Decisions about priorities

Substantiative Criteria e.g. Severity of the disease (taking into account accessible treatment), Prevalence of the disease

Procedural Criteria e.g. Accountability for Reasonableness Standard

Stakeholders e.g. Experts, patients, politicians



(Unmet) Medical Need – Take home messages

- Unmet medical need: vague and heterogeneous understanding
- Medical Need – clarification of empirical and normative tasks
- Setting priorities with reference to needs: Substantiative and procedural criteria needed

