EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Unmet medical need; definitions and need for clarity

EMA-Payer community meeting
19 September 2017

Definition of unmet medical need (regulatory side)

Conditional marketing authorisation:

- → for products where the benefit-risk balance is such that the immediate availability outweighs the limitations of less comprehensive data than normally required, i.e. medicines with an established potential to address an unmet medical need.

- Article 4 paragraph 2 of Commission Regulation (EC) No. 507/2006 specifies that unmet medical needs mean a condition for which ***there exists no satisfactory method of diagnosis, prevention or treatment in the Union*** *or, even* ***if such a method exists****, in relation to which the medicinal product concerned will be of* ***major therapeutic advantage*** *to those affected*.

Accelerated assessment:

- → medicinal products of a major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation

- This should be justified by the applicant: typically, the justification could present the arguments to support the claim that the ***medicinal product addresses to a significant extent the unmet medical needs*** for maintaining and improving the health of the Community, ***for example, by introducing new methods of therapy or improves existing ones***.

Other areas where unmet need is considered

Orphan designation

- Article 3 of Regulation 141/2000 refers to life-threatening or chronically debilitating nature of the condition as requirement for orphan designation of a medicine
- unmet need implicit in significant benefit criteria for designation: responsibility of the sponsor to establish that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question, or if such method exists that the medicinal product will be of significant benefit to those affected by that condition.

PRIME

- unmet medical need definition concept "borrowed" from CMA and applied in context of fostering accelerated assessment

Paediatric investigation plans

- Can be waived if the medicine does not represent a significant therapeutic benefit over existing treatments for paediatric patients (definition similar to the one used for orphan medicines)

Some issues that regulators have to address

Patient vs population focus for unmet medical need

- "Last line" unmet need might always be justified

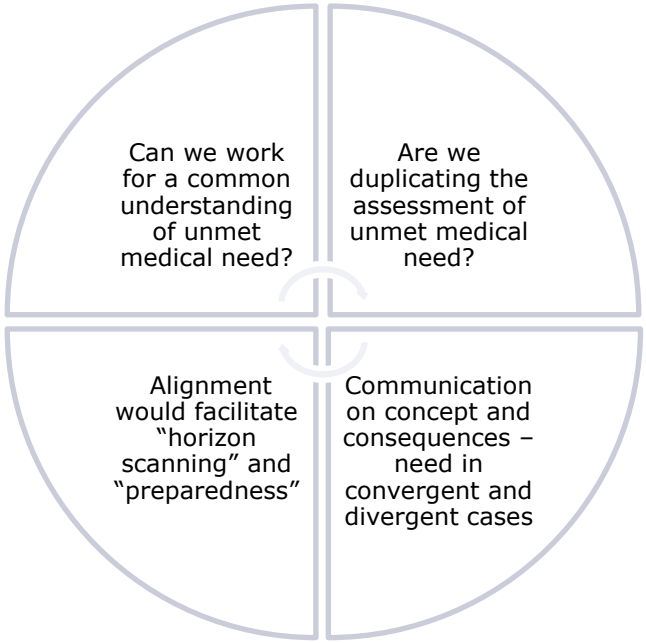
Unmet need

- Binary decision
- Not quantified – no degrees

Medicine focus (how it addresses need) and not relative - compared across medicines

No prioritisation of procedures, some (minimal) prioritisation of resources (accelerated, PRIME)

Topics for discussion



For discussion

UMN is area of common interest for regulators and payers, specially due to its sensitivity but also due to its potential for interpretation

Multi-stakeholder involvement needed in discussions

Are we ready to work for an agreed definition or compatible ones?