UNMET MEDICAL NEED IN RARE AND PAEDIATRIC DISEASES

12th STAMP meeting

29 January 2021

Pharmaceutical strategy

- Fulfilling unmet medical needs and ensuring accessibility and affordability of medicine
  - **Prioritising unmet medical needs**: investment does not necessarily focus on the greatest unmet needs, due to the absence of commercial interest or limitations of the science
    - **Orphan and paediatrics:** reflection on how to tailor the system of incentives provided by the EU pharmaceuticals framework better in order to stimulate innovation in areas of unmet medical needs starting from O/P
    - **Antimicrobials**
    - **Increased cooperation** in scientific advice and convergence on key concepts, such as 'unmet medical need'
    - Reflection on how to tailor the system of incentives provided by the EU pharmaceuticals framework better in order to stimulate innovation in areas of unmet medical needs

Revision of the pharmaceutical legislations

- Roadmap inception impact assessment Q1 2021
- External stud(ies) on the implementation (launch of process Q2 2021 – final report Q1 2022)
- Public consultations Q3 2021
- Adoption of proposal Q4 2022

Throughout the process: work closely with members of pharmaceutical committee and in other fora

Revision of the orphan and paediatric Regulations

- Joint Evaluation of the two Regulations (summer 2020)
- Inception Impact Assessment (Roadmap)
  - Feedback mechanism (25 November 2020 → 6 January 2021)
  - Over 110 replies received
- Impact Assessment study: 1Q-3/4Q 2021
  - Supported by an external study (1Q-3Q 2021)
  - OPC and targeted consultations foreseen
- Legislative proposal 1Q 2022

Summary of problems found in evaluation

- Insufficient development in areas of greatest unmet medical needs
  - 95 % rare diseases no treatment option
  - Developments do not address highest needs for children
  - 'One-size-fits-all' incentives and rewards <-> unmet needs
- Availability and accessibility varies across MS
  - No link between incentive and placing on market (orphans)
  - Limited generic competition after expiry of exclusivity periods
- Scientific and technological developments cannot be fully exploited
  - Instruments in legislation not adequate for advances in science: biomarkers and personalised medicine
  - Exclusion from obligation to conduct PIPs (mechanism of action)
- Certain procedures inefficient and burdensome

Objectives of the revision

- To foster research and development of medicines for rare and paediatric diseases in areas of unmet need and in better alignment with patient needs
- To ensure availability and timely access of patients to orphan and paediatric medicines
- To ensure legislation to be fit to embrace technological and scientific advances
- To provide effective and efficient procedures, for assessment and authorisation

Impact assessment study

- **Paediatric Option 2:** the reward in form of a 6-month SPC extension will be limited only to products fulfilling areas of **unmet paediatric therapeutic needs**
- **Paediatric Options 3 and 4:** introduce a new reward for **unmet needs for children**
- **Orphan options 3 and 4:** for products addressing an **unmet need and for rare paediatric diseases**, we will explore novel incentives that complement or replace the market exclusivity.

Feedback on UMN – Member states

- Clear and common understanding as well as an unambiguous definition with transparent criteria at the EU level, coherent with the actions in the pharmaceutical strategy and in the orphan and paediatric regulations
- Currently, “unmet medical need” is used as a binary category (yes/no), while a quantification of the unmet need may be necessary as a basis for an award of graduated incentives.
- Rare diseases = unmet medical need
- How much the additional benefit/added-value to consider it as responding to an unmet medical need.
- Root causes of unmet medical needs: the scientific knowledge of the disease is lacking or that the market is unattractive? Different root causes will need different solutions.

Feedback on UMN – stakeholders

- **NGOs/patients**: Unmet needs are implicit in the 'significant benefit' criteria for designation. An explanation on satisfactory method is expected. A legally binding definition could raise more problems than it would solve. It is preferable to ensure that early dialogue takes place at a very early stage, on a specific disease, in a multi-stakeholder format.

- **Academia**: It is key to get a multi-stakeholder endorsed definition of 'unmet medical need' to avoid a too strict definition.

- **Industry**: The absence of any treatment is not the only unmet need to consider. Disease severity, burden of illness and impact on the quality of life of patients, families and carers are also essential elements. A multi-stakeholder involvement is needed to overcome the narrow setting of conditional marketing authorization and include scientific and societal evaluation.

Criteria for UMN in orphans and paediatrics

Group discussion

- **Aim:** to agree on an outline of criteria which will be explored in the Impact Assessment study
- One hour discussion, rapporteur appointed

General discussion

- **Aim:** to discuss and validate the draft criteria
- Rapporteurs present the outcome of the group discussion

Group discussions

- **Paediatric Group:** please stay in this Webex

- **Orphan Group:** please go to the second Webex link

THANK YOU!