EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems, medical products and innovation
Medicines: policy, authorisation and monitoring

STAMP 12/50

Summary record

STAMP Commission Expert Group

29 January 2021

12th meeting

Summary Record

The Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) held its 12th meeting on 29 January 2021, chaired by Unit B5 - Medicines policy, authorisation and monitoring of Directorate-General for Health and Food Safety. Representatives from 24 Member States and the European Medicines Agency (EMA) participated in the meeting.

1. ADOPTION OF THE AGENDA

The draft agenda was adopted and the Commission opened the meeting and welcomed participants.

2. INTRODUCTION TO THE DISCUSSION AND FEEDBACK ON THE INCEPTION IMPACT ASSESSMENT

The Commission introduced the discussion and explained the links between the inception impact assessment and the EU pharmaceutical strategy including the broader picture on unmet medical need (UMN).

The objectives of the revision of the orphan and paediatric Regulation are for the most part common also to the pharmaceutical strategy, including the need to fulfil unmet medical needs of patients and ensure accessibility and affordability of medicines. While today's discussion aimed exclusively at supporting the impact assessment for the revision of the two legislations, a wider discussion on unmet needs will take place in the framework of the pharmaceutical strategy.

The timeline for review of the orphan and paediatric legislation was outlined (legislative proposal should be ready by Q1 2022). The Commission briefly explained that the aim of today's discussion is to inform the impact assessment on the revision of the two legislations.

The Commission explained that one of the main problems identified in the evaluation of the orphan and paediatric legislation is the insufficient development in areas of great UMN for patients. One of the objectives of the revision of the two Regulations is therefore to foster development and authorisation of medicines for rare diseases and for children, especially in these areas of unmet need and in better alignment with patient needs.

The Commission reminded of the recent publication of the inception impact assessment (IIA), which outlines possible solutions for the review of this legislation. It briefly outlined the feedback from Member States and stakeholders on unmet medical needs received during consultation.

The focus of today's discussion is on criteria for UMN in rare and paediatric diseases. A separate future discussion may focus on the system of incentives linked to unmet medical need addressed by a specific orphan or paediatric medicine.

Interventions/comments:
Member States welcomed the discussion on UMN, but also indicated the need for a broader discussion on UMN. It was mentioned that there is a discrepancy in the UMN definitions depending on areas. It was stressed that it should also be looked if a product meets the defined UMN and the kind of incentive that can be provided to it in such case. It was mentioned that the discussion on incentives should not influence the discussion on the definition of UMN.

The Commission clarified that this specific discussion on UMN will inform the testing of options in the impact assessment for the revision of the orphan and paediatric legislation. It will also inform the broader discussion on UMN in the framework of the Pharmaceutical Strategy for the EU. The wish is to keep in the discussion, when possible, separated the criteria of UMN (*what*) and the incentives (*how*).

The participants split into 2 parallel groups.

3. DISCUSSIONS IN GROUPS – PAEDIATRICS

The Commission introduced the topic. There have been good developments in paediatric medicines, but the developments generally follow adult development (little innovation has taken place so far in purely paediatric indications).

Subsequently, the discussion focused on alternative solutions to boost development in cases where an adult development is ongoing. If there is already an adult development, the mechanism of action of the product may be useful to treat a different paediatric disease.

However, in case there is no adult development ongoing, it was discussed in which cases the 'paediatric only' developments should be further incentivised. The idea of creating a list of therapeutic needs was discarded by several participants.

4. DISCUSSIONS IN GROUPS – ORPHANS

The Commission introduced the topic and explained that the focus is on criteria for UMN for orphans.

The discussion that followed narrowed down to two sets of criteria to define UMN: 1. 'seriousness of the disease'; 2. the 'availability of treatments'. The availability of only symptomatic treatments was discussed. Furthermore, it can also be the case that there is no available treatment adapted to a specific subpopulation.

The importance of having separate discussions on the definition of what is UMN and on how a product addresses this UMN was highlighted.

5. FEEDBACK FROM GROUPS AND GENERAL DISCUSSION

Feedback from paediatric group by rapporteur:

Concerning the discussion on mechanism of action, it was mentioned that a marketing authorisation holder may not have the expertise in all therapeutic areas and the need to cooperate with academia was mentioned.

The following issues were also raised in the feedback:

- When to incentivise and if incentives should be graduated?
- If availability of the right type of formulation and/or availability only in a few Member States should also be taken into account when discussing UMN.
- Issues around pricing and reimbursement were mentioned, but it was also concluded that these areas are *outside* the scope of the paediatric legislation.
- Discussion about UMN in paediatric area and the link to the EU pharmaceutical Strategy.
- The difficulty about how to compile a list of priorities.

Feedback from orphan group by rapporteurs:

General considerations were outlined around two main sets of criteria that should be used to define UMN in the area of rare diseases:

1. Seriousness of disease
2. Available treatments

General discussion

The discussion focused on the availability of treatments. The Commission raised the issue of variation in availability between Member States and how this can be taken into account in relation to an UMN. It was discussed if availability is a criterion for UMN. It was also discussed that, when a product in a specific therapeutic area is given incentives, this may influence its availability on national markets. This availability could lead to gradual incentives. The incentives may also be adapted to the *level* of UMN addressed. The use of magistral preparations and off-label use was also discussed. These aspects should be further considered in the Impact Assessment.

Future activities of STAMP

The Commission concluded that the elements discussed in the meeting will be presented during the next Pharmaceutical Committee meeting. Following this presentation, it will be considered if another discussion in a STAMP meeting may be needed.

The next STAMP meeting is tentatively planned for 12 March 2021 (tbc).

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