EUROPEAN COMMISSION
HEALTH AND FOOD SAFETY DIRECTORATE-GENERAL

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

Pharm 815

PHARMACEUTICAL COMMITTEE
11 December 2020

Subject: Revision of the orphan and paediatric medicines legislation

Agenda item 2

1. INTRODUCTION

First, we would like to present the inception impact assessment for the revision of the EU legislation on medicines for children and rare diseases[1] (**see below under 2**). Second, we would like to continue the discussion we started last year on setting criteria to define "unmet medical need" in the areas of rare and paediatric diseases (**see further below under 3**).

2. DISCUSSION POINT: INCEPTION IMPACT ASSESSMENT

The Commission will present the main problems found in the evaluation of the medicines for rare diseases and children legislation,[2] the objectives for revision and the options identified in the inception impact assessment. We would like to invite the Pharma Committee members to express their views on the options presented. The Commission is planning to launch an Impact Assessment study in February 2021.

3. DISCUSSION POINT: CRITERIA FOR UNMET MEDICAL NEED

Background

In the inception impact assessment on the revision of the EU legislation on medicines for children and rare diseases, it was emphasised that one of the four main problems identified in the evaluation of the legislation is the **insufficient development in areas of greatest unmet medical needs** for patients.

Findings in the evaluation showed that the Regulations have not boosted developments in all major therapeutic areas. It was found that 95% of rare diseases still have no treatment options. It was also found that in areas where the therapeutic needs of children and adult diverge, product development for these "rare paediatric diseases" has been limited.

The reason for these great unmet medical needs comes from insufficient research and low interest from developers in investing in some of these therapeutic areas.

[1] https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12767-Revision-of-the-EU-legislation-on-medicines-for-children-and-rare-diseases
[2] https://ec.europa.eu/health/human-use/paediatric-medicines/evaluation_en

Furthermore, both Regulations have been built around "one-size-fits all" incentives and rewards, which do not always provide an adequate tool to stimulate developments in areas of unmet needs.

In a future revision of the Orphan and Paediatric Regulation, it will be considered to design specific rewards and incentives to direct medicines' development in specific areas of pressing needs for patient suffering from rare diseases and for children.

Although the discussion about unmet medical need is broader than rare and paediatric diseases alone, agreement on criteria for unmet medical needs in rare diseases and children would be an urgent first step also in view of the revision of the legislation for rare diseases and children.

We therefore would like to continue the discussion on unmet medical needs that we already started in the Pharmaceutical Committee last year.[3] In order to have a fruitful meeting on December 11, we would like you to reflect beforehand on a number of questions.

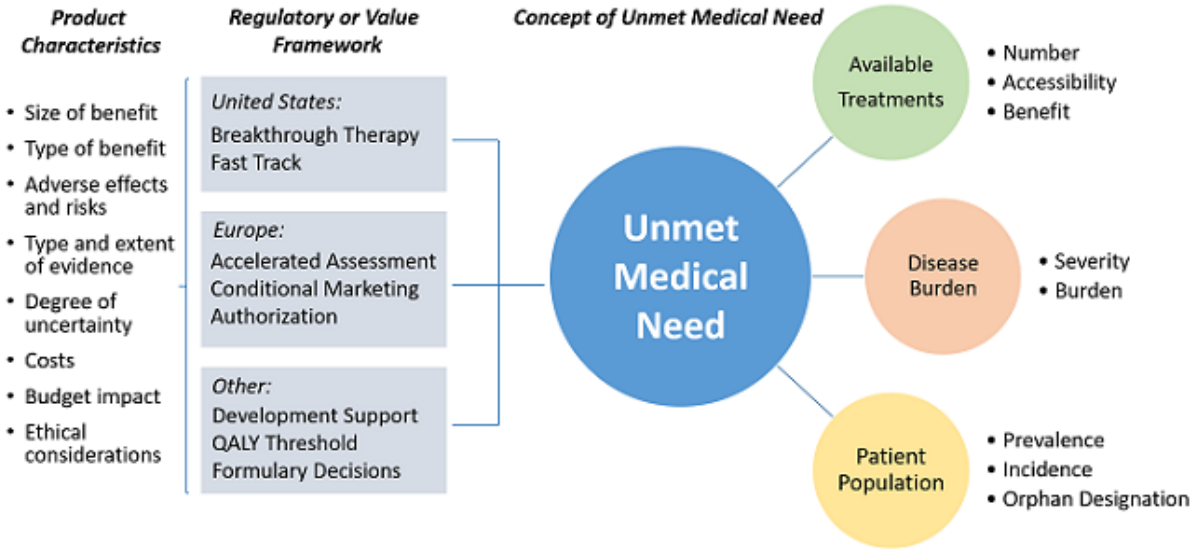
Questions and discussion

Medicines for rare diseases

1. Do we need to agree on new 'one-size-fits-all' criteria for unmet needs in rare diseases or would we first need more clarity on the current criteria?

Current criteria relate to:

- size of the affected population (prevalence)
- availability (and adequacy) of treatments
- disease severity or burden



Ref. DIA

[3] Meeting of Pharma Committee of 17 December 2019 and document Pharm 785.

Medicines for children

1. Article 43 of the Paediatric Regulation[4] provides criteria to establish an inventory of therapeutic needs of children:
    a. Could such criteria be the base to set up a system to "designate" developments, which could address unmet needs of children?
    b. Would it be possible to be more specific with such criteria?

2. Alternatively, would it be conceivable to come up and keep updated a list of paediatric unmet needs? Could such a list be based on the criteria above? How would such a list look like?

Future forum for discussion and way forward

The objective of the 11 December meeting will thus be to start a discussion on how to further define criteria to determine unmet needs for patients suffering from rare diseases and for children.

For more detailed discussion and reflection, we would like to propose using STAMP as a forum that will meet in January/February 2021 for follow-up discussion in order to further reflect on those criteria and to inform the Impact Assessment study. If need be, STAMP would also have the ability to bring different constituents around the table. We would appreciate your views on who should be involved in those discussions.

[4] "In establishing the inventory of therapeutic needs, account shall be taken of the prevalence of the conditions in the paediatric population, the seriousness of the conditions to be treated, the availability and suitability of alternative treatments for the conditions in the paediatric population, including the efficacy and the adverse reaction profile of those treatments, including any unique paediatric safety issues, and any data resulting from studies in third countries."