Open Public Consultation on the revision of EU rules on medicines for children and rare diseases

Introduction

The EU rules on medicines for rare diseases and medicines for children were adopted in 2000 and 2006, respectively. The rules were designed to improve the treatment options available to 30 million European patients affected by one of over 6000 rare diseases, as well as for 100 million European children affected by paediatric diseases. At the time, there were limited or no medicinal products available for treatment of both groups.

A recent evaluation of the rules showed that they have stimulated research and development of medicines to treat rare diseases and other conditions affecting children. However, the evaluation also revealed shortcomings in the current system. The rules have not been effective for stimulating the development of medicines in areas of unmet needs (e.g. 95% of rare diseases still have no treatment option), and they have not ensured that the medicines are accessible to all European patients across all Member States.

The rules provide incentives and rewards, and their design can influence business decisions on research and development for new medicines, as well as whether such investment can be focused in areas of the greatest need for patients. In addition, the system of incentives can impact market competition and indirectly influence the availability of and access to those medicines by EU patients.

About you

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- Other

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* Scope
  - International
  - Local
  - National
  - Regional

* Level of governance
  - Local Authority
  - Local Agency

* Level of governance
  - Parliament
  - Authority
  - Agency

* Organisation name
  *255 character(s) maximum*

* Organisation size
  - Micro (1 to 9 employees)
  - Small (10 to 49 employees)
  - Medium (50 to 249 employees)
  - Large (250 or more)
Transparency register number

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- Senegal
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* Language of my contribution
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  - Dutch
  - English
  - Estonian
  - Finnish
  - French
  - German
  - Greek
  - Hungarian
  - Irish
  - Italian
  - Latvian
  - Lithuanian
  - Maltese
  - Polish
  - Portuguese
  - Romanian
  - Slovak
  - Slovenian
  - Spanish
  - Swedish
Questionnaire on the revision of EU rules for medicines for rare diseases and children

Q1: The main problems identified in the evaluation of the legislation for medicines for rare diseases and for children were the following:

- Insufficient development in areas of the greatest needs for patients.
- Unequal availability, delayed access, and often unaffordable treatments for patients in the EU Member States.
- Inadequate measures to adopt scientific and technological developments in the areas of paediatric and rare diseases.

In your opinion, are there any other barriers to the development of treatments for rare diseases and children?

2000 character(s) maximum

Q2: In your opinion, and based on your experience, what has been the additional impact of COVID-19 on the main problems identified through the evaluation? Is there a 'lesson to be learned' from the pandemic that the EU could apply in relation to medicines for rare diseases and children?

2000 character(s) maximum

Q3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients?

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When considering whether a particular medicine is eligible for support, the rarity of the disease – the total number of cases of a disease at a specific time, currently less than 5 in 10 000 people – forms the main element of the EU rules on medicines for patients suffering from rare diseases.
Some diseases occur frequently, but last for a relatively short period of time (for example, some rare cancers). These are covered by the EU rules on medicines for rare diseases and the principle of rarity. However, because many patients acquire such diseases during a specified, limited period of time, those diseases should not be considered as rare in the EU anymore.

Amongst all medicines for rare diseases which become available to the EU patients, only those bringing a clear benefit to patients should be rewarded. Clear rules should apply to decide if one medicine brings a clear benefit to patients when compared to any other available treatment in the EU for a specific rare disease.

Additional incentives and rewards should exist for medicines that have the potential to address the unmet needs of patients with rare diseases, for example in areas where no treatments exist.

Other (please suggest any other criteria/approaches you think might be relevant).

Q4: What factors are important to take into consideration when deciding if one medicine for a rare disease brings more benefits compared with other available treatments?

Q5: What do you consider to be an unmet therapeutic need of rare disease patients and children?

- Authorised medicines for a particular rare disease or a disease affecting children are not available, and no other medical treatments are available (e.g. surgery).
Treatments are already available, but their efficacy and/or safety is not optimal. For example, it addresses only symptoms.

Treatments are available, but impose an elevated burden for patients. For example, frequent visits to the hospital to have the medicine administered.

Treatments are available, but not adapted to all subpopulations. For example, no adapted doses and/or formulations, like syrups or drops exist for children.

Other (please specify).

2000 character(s) maximum

Q6: Which of the following measures, in your view, would be most effective for boosting the development of medicines addressing unmet therapeutic need of patients suffering from a rare disease and/or for children? (1 being the least effective, 10 being the most effective)

at most 1 answered row(s)

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<td>Additional scientific support for the development of medicines from the European Medicines Agency</td>
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<td>Assistance with authorisation procedures, such as priority review of the application from the European Medicines Agency and/or expedited approval from the European Commission</td>
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Do you have other suggestions that would allow the EU to boost the development of specific medicinal products?

2000 character(s) maximum

Do you see any drawbacks with the approaches above? Please describe.

2000 character(s) maximum

Q7: Which of the following options, in your view, could help all EU patients (irrespective of where they live within the EU) to provide them with better access to medicines and treatments for rare diseases or children?

- Greater availability of alternative treatment options. For instance, by allowing a generic or biosimilar product to enter the market faster.
- Allowing companies that lose commercial interest in a rare disease or children medicine product to transfer its product to another company, encouraging further development and market continuity.
- For companies to benefit from full support and incentives, products need to be placed timely on the market within all Member States in need as soon as they received a marketing authorisation.

Other (please suggest any other solution you think might be relevant).

2000 character(s) maximum

Q8: Most of the medicines for rare diseases are innovative medicines. However, in some cases, an older, well-known medicine for a common disease can be repurposed (i.e., using existing licensed medicines for new medical uses) to treat a rare disease. In your view, what would be the appropriate way to award innovative medicines in cases where other treatments are available:

- Both new, innovative medicines and well-known medicines repurposed to treat a rare disease should receive the same reward
Q9: Despite the presence of a dedicated procedure (the Paediatric Use Marketing Authorisation, PUMA) in the Paediatric Regulation, many older medicines that are currently used to treat children have only been studied for use within adult populations, and therefore lack the appropriate dosage or formulation suitable for use in younger patients. However, the development of medicines that have been adapted for use in children could also result in a product being more expensive than its adult-focused counterpart. In your view:

Should the development of appropriate dosage or formulation suitable for children of such older medicines be stimulated even if their price will be higher than that of the available alternatives?

- Yes
- No
- Do not know/cannot answer

Please explain your answer.

2000 character(s) maximum

How would you suggest stimulating further development of appropriate dosage or formulation suitable for children of such older medicines?

2000 character(s) maximum

How can it be ensured that such developed products are reasonably profitable for companies and also reach patients?

2000 character(s) maximum