

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

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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.

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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?

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The EU framework has successfully driven medicine development for rare and paediatric diseases and a promising pipeline. The lengthy timeline of OMP development can delay the positive effects of the legislation to be fully evident. Two thirds of developed OMPs treat the 390 most prevalent conditions. Novel solutions could further encourage development for the 95% of rare diseases with little or no available treatment. EuropaBio encourages the Commission to consider the following elements for the future:

Lack of scientific progress: The observed insufficient development in some areas should be contextualized, as most conditions falling within the 95% are extremely rare and lack basic scientific understanding. R&D is both complex and economically challenging.

Burdensome regulatory framework: Developers (particularly SMEs) often have to discuss and negotiate with different EMA committees (CHMP, COMP, PDCO). We need an EMA “one-stop-shop” for OMP and paediatric development. RWE should be accepted to match the evidence gaps inherent to conditions affecting small patient populations. The lack of predictability on the maintenance of the OMP status at the time of approval should be addressed.

Commercial uncertainties: The EU market is unpredictable for developers and uncertain for investors, due to the different interpretation of value among Member States (in some cases, as in Italy, even within a single Member State due to a regionally managed NHS). The fragmented access pathways do not provide clarity as to when and where treatments will actually reach patients, discouraging R&D. This is evident in the lengthy national P&R processes, differences in value assessment processes/requirements, lack of health system readiness/clinical expertise to assess OMP, payer reluctance to accept “unproven” products or RWE. Medicines for children are also affected by e.g off-label use of the adult product and lack of willingness to pay more for paediatric formulations.

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?

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The pandemic has shown that, when all factors come together, Europe is capable of discovering and developing life-saving innovation fast: decades of previous research have allowed deployment of the right technology to tackle COVID; decentralized clinical trials have helped to make R&D more efficient; rolling reviews and faster timelines have allowed to get these products rapidly approved; and intellectual property has allowed for hundreds of partnerships needed to transform science into products and to ramp up production.

The development of orphan and paediatric medicines could benefit from the expedited assessment timelines we have witnessed in COVID times, through swift and proactive engagement from the EMA and collaboration between the scientific committees and working parties.

The value of using digital tools and harmonized flexibilities has been proven. The development of electronic product information can improve access to up-to-date product information.

EuropaBio would welcome considering the adoption of innovative regulatory approaches tested during the COVID-19 pandemic response by the EMA and the EU network of medicines agencies. In particular, the possibility of aligning EMA and FDA on paediatric development was a welcome approach that should be continued post-COVID. Regulatory convergence is key when developing globally, as is the norm for rare and paediatric diseases.

The EU should lead in implementing an agile regulatory system which allows for adaptable and accelerated regulatory assessments to fulfil unmet medical need. The experience of the EMA during the pandemic (e.g. rolling reviews of incoming scientific evidence to speed up assessment) must inform future regulatory tools. We realise that this needs to be supported by increased resources in the European Network which we would fully support.

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

at most 4 answered row(s)

	Very adequate	Moderately adequate	Not at all adequate
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.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>

<p>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 <u>not</u> be considered as rare in the EU anymore.</p>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
<p>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.</p>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
<p>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.</p>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>

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

2000 character(s) maximum

EuropaBio agrees with the statement above that the current prevalence threshold of 5 in 10,000 is adequate. The overwhelming majority of the 6000-8000 known rare diseases are identified in less than 100 people globally. On the other hand, the 5% of the most prevalent rare diseases affect about 80% of patients. The 95% figure of non-addressed rare diseases is also an underestimation of the real unmet medical needs, as “very few” existing treatments are curative. For most rare diseases, such as haemophilia, cystic fibrosis, or seizure disorders, people continue to live with significant disease burdens.

It is EuropaBio’s position that no rare disease patient should be prioritised or de-prioritised based on the duration of their illness, which in the case of rare cancers, can be fatal.

On the 3rd option above, EMA should interpret significant benefit as it is virtually impossible to set rules for every scenario. There are also treatments where endpoints may not be “well defined”, and consequently, demonstrating clinical benefit is not clear cut. This is also a challenge given typical trial designs, e.g. single arm, small sample. This does not take into account that, for the most part, there are no other available treatments, so comparison is not possible.

EuropaBio agrees that additional incentives and rewards should exist for medicines that have the potential to address the unmet needs of patients with rare diseases, also in areas where no treatment currently exists. However, we emphasise that until a condition is curable, unmet needs for these patients remain and that even if a treatment for a condition exists, it does not mean that the patient’s condition or quality of life is optimal. The EU should increase the scale and continuity of funding for basic research and early development, building on existing tools (such as EJP RD) and going beyond (e.g., public-private partnerships with funding conditionality to address diseases without effective treatments

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?

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There are robust significant benefit provisions in the EU framework. Key elements to be taken into account when discussing unmet needs include:

Adequacy of available treatments: Treatment burdens exist also for available treatments and multiple patient populations can be affected differently (as to age, gender, etc.) Some subgroups of patients lack authorised treatments (e.g. due to immunogenicity). Diseases also lack satisfactory treatments where significant mortality/morbidity remains and new innovative therapies show considerably better efficacy/safety vs authorised treatments. Examples of rare disease with significant disease burdens despite existing treatments are haemophilia, cystic fibrosis, seizure disorders. Another dimension are the side-effects induced by the treatment itself.

Disease severity or burden: Both mortality rates and quality of life/burden of disease/burden of available treatment should be considered for UMN. Disease severity or burden are relative to the specific disease and cannot be compared to one to another. Patients are best placed to know when they are suffering an UMN and should be consulted.

Population-based considerations: Low prevalence is not a good indicator of UMN. E.g., there is significant unmet need in Alzheimer's disease. For rare conditions in particular, all prevalence under 5 in 10000 people should be considered.

The EU should work with national authorities to encourage the acceptance of "significant benefit" in P&R discussions, as divergence and fragmentation of benefit evaluations among Member States create major disruption in equity of access of patients to innovative products. Heterogeneous national HTA and P&R procedures contribute to a lack of alignment between developers, payers, clinicians, and patients' needs on the benefit of treatments. This creates uncertainties on willingness to pay for OMPs.

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

On the first option above, we agree that the unavailability of an authorised medicine to the patient who needs it gives rise to a situation of UMN, but not in settings of e.g. malfunctioning cross-border healthcare or delayed P&R decision.

EuropaBio highlights that the concept of UMN already exists in the current EU legal framework, e.g. in Article 14(a) of Reg 726/2004 and Article 4(2) of Reg 507/2006. Furthermore, the concept of “major therapeutic advantage” can be found in Art 14 (11) of Reg 726/2004; Art 3 (1)b of Reg 141/2000, Art 8 (3) of Reg 141 /2000, Art 6 (2) and 11.1 (c) of Reg 1901/2006, and Art 14 (9) of Reg 726/2004.

All the concepts above contain 3 key elements: 1) improved efficacy, 2) improved safety, or 3) major contribution to patient care.

The IIA seems to suggest the development of criteria to determine UMN, plus a system for identifying products addressing UMN. Overall, EuropaBio feels the options under consideration suggest a very narrow understanding of where UMN lie and seems to focus on new molecular entities for diseases without existing treatments, rather than focusing on addressing the UMN.

EuropaBio would like to reiterate and stress that any intervention which can improve quality or length of life, ease of treatment administration, or reduce treatment burden, disease severity, morbidity, mortality, or any other complications, should be considered as meeting an UMN. A suitable level of flexibility should be ensured, as the concept of UMN may evolve over time due to evolution of treatments, science, and disease understanding, hence there may be a drug life cycle approach taken. Clarity will also be essential to avoid prolonged disputes about whether or not the definition is met in a given circumstance. May also consider effects on caregiver and the healthcare system.

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 4 answered row(s)

	1	2	3	4	5	6	7	8	9	10
Assistance with Research & Development (R&D), where medicines under the development can benefit from national and/or EU funding	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>						
Additional scientific support for the development of medicines from the European Medicines Agency	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>					

Assistance with authorisation procedures, such as priority review of the application from the European Medicines Agency and/or expedited approval from the European Commission	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>				
Additional post-authorisation incentives that complement or replace the current incentives and rewards	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Do you have other suggestions that would allow the EU to boost the development of specific medicinal products?

2000 character(s) maximum

There are essentially 3 reasons why products are not developed and we propose solutions on each:

Lack of scientific progress: Public-private cooperation could help, e.g. more funding for basic research and data collection/analysis infrastructure, esp. for paediatrics; dedicated PPPs on ultra-rare diseases; utilization of EHDS for rare and paediatric diseases. The ERNs should be strengthened and further expanded.

Market failure: More incentives should be considered for ultra-rare diseases, e.g. transferable market exclusivity vouchers would allow a company to invest in products that do not have commercial viability on their own. A priority review voucher could help, but only if it is linked to faster HTA and P&R procedures. Working with national authorities to encourage the acceptance of “significant benefit” in P&R discussions (as in Germany) would help to overcome uncertainty in investment decisions. Current incentives have supported OMP development for a significant number of patients and should be maintained. We support additional post-authorisation incentives which complement the current incentives or rewards.

Fit-for-purpose regulation: Better predictability, efficiency of the regulatory review, expedited pathways (e.g. rolling reviews) and acceptance of innovative clinical trial designs are needed. Patient scarcity in rare and paediatric diseases requires a global approach and innovative evidence generation. Both regulators and industry should enable innovation in complex clinical trials. Harmonisation could support incorporation of digital technologies into trials. RWE for regulatory assessment should be accepted to drive clinical development programmes. A common EHDS could become an important tool to boost development of new medicines in a sector such as rare diseases. Improvements to regulatory procedures are greatly needed, but true success requires an end-to-end approach supporting R&D before and market access after the regulatory step.

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

2000 character(s) maximum

EuropaBio believes that the proposals for improvement should address properly the gaps and issues identified and target the underserved rare diseases with a clear strategy. The incentive regime has brought considerable benefits to society, but this does not mean that the environment for OMPs cannot be improved. For EuropaBio, there are two sets of issues to be addressed – the 5% of rare diseases where the number of patients is the greatest where the framework can be marginally improved and the 95% of very rare diseases that require different thinking with new solutions.

Rare diseases with a prevalence of less than 5 in 10'000 represent 5% of the rare diseases but about 80% of the rare diseases patients. Unmet needs still persist in these areas despite existing treatments. For these most prevalent diseases improvements should concentrate on:

1) regulatory improvements including enhanced predictability and higher efficiencies of regulatory reviews, simplification of processes and expedited pathways to support innovation

2) improved access solutions like single access pathways that award automatically additional therapeutic value to orphan medicines or make better use of RWE infrastructures and improving cross border healthcare for patients with innovative financing mechanisms for advanced therapies

3) incentives based on the current orphan designation definition supported by current market exclusivity conditions

For the other 95% of very rare conditions representing less than 20% of the patients, novel solutions are needed. The development of treatments for these conditions require a very different economic model. Strong research push solutions like specific funds for basic research and data infrastructure or PPPs and market pull incentives, such as transferrable vouchers including dedicated EU-level R&D funding.

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

EuropaBio acknowledges that generic and biosimilar entry into the market can encourage competition in some cases. The low number of rare disease patients does not attract generic or biosimilar developers. In practice, enabling faster market entry for these products will not necessarily encourage their development.

On the second point above, developers of OMP and paediatric medicines are currently already free to transfer their assets to another entity and this would not improve access.

Finally, it is the innovative biopharmaceutical companies' general interest to make their products available to as many patients, in as many countries and as early as possible. The process of placing a product on the market is not entirely in the control the company, but also the respective Member State. For many companies, particularly SMEs, preparing the relevant dossiers for multiple HTA and payer bodies with varying requirements across several Member States represents a significant administrative and financial burden. These can include lengthy & bureaucratic P&R negotiation processes, differences in value assessments among Member States, national access timelines (incl. supplementary requirements at regional level within a country), lack of health system readiness, payer reluctance to accept unproven products or the use of non-clinical trial data and launch sequencing related to IPR. A product cannot be available across the EU at the exact same time, even if P&R applications are filed the same day. Moreover, imposing such an obligation to the industry overall would unfairly discriminate SMEs with limited resource capacities.

Solutions could include local acceptance of the additional therapeutic value of OMPs, and medicines for children, expedited access pathways (e.g. in Germany), increased alignment between regulatory and HTA bodies, improved acceptance of RWE, earlier dialogue between companies and payers, and improved cross-border healthcare for rare disease patients.

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
- New, innovative medicines to treat a rare disease should receive an enhanced reward
- Do not know/cannot answer

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

Developing a paediatric indication for a product requires demonstrating the same quality, safety, and efficacy requirements as for adults, for a population with specific biological and physiological differences, thus requiring a separate development plan. For this reason, incentives for this investment undertaking are of high importance. When the compound is off patent, as for a PUMA, regardless of price, the incentive offered (regulatory data protection) is not sufficient as existing generic medicines continue being used and reimbursed.

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

2000 character(s) maximum

Novel incentives and public funding to support SMEs, academia, may be needed to provide the necessary stimulus for PED formulations of off-patent products. Tailor-made incentives should be provided for companies that, since the first phases of research and development, work to adapt the same product's dosage or formulation to both adult and children populations. However, this development will only be successful if it is accompanied by measures at Member State level to ensure that such dosage or formulation are then prescribed, used and reimbursed in the Member States. Such an action could also help the PUMA scheme to be more usable.

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

2000 character(s) maximum

EuropaBio does not believe that what constitutes "reasonable profit" falls within the competences of the EU, which should rather focus on the value provided by the medicine. In relation to the question specifically, if there are no complementary national push incentives and willingness to pay for additional new formulations by Member States, there is a high risk that any push incentives and additional funding to support R&D might not lead to the results hoped for. It must be ensured that products are prescribed and reimbursed, and this remains primarily a national competence.

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