

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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* Organisation name

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Association of European Cancer Leagues (ECL)

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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?

2000 character(s) maximum

It is important to consider how incentives steer innovation across the various therapeutic areas. The problem is not around the barriers to the R&D of treatments for rare diseases as a whole but for specific disease areas that do not get enough attention. Here below our views on the scientific, financial, and regulatory hurdles:

- **Scientific hurdles:** small population sizes of rare disease patients affect the extend the biological mechanisms are known. To cover these knowledge gaps, alternative clinical trials scientifically valid should be considered and academia and public institutes may be the solution. The small patient populations means that it is difficult to meet the rigorous scientific demands required by authorities/HTA bodies. Hence, there is a need for developing trial designs that can accommodate the inherent low patient populations of rare diseases/specific tumor mutations. For many rare tumors, survival figures are still very low.
- **Financial hurdles:** One-fits- all incentive framework is not adequate as the SWD highlights at page 14: "The current regulatory framework for the EU Orphan Regulation does not contain any provision to safeguard the affordability and accessibility of products even when no significant R&D investments have been made by the sponsor. Applications such as those for well-established use products and known active substances receive the same reward as new active substances".
- **Regulatory hurdles:** The administrative burden associated with the work performed by COMP members is not sustainable in the long-term. Also, the differences with the interpretation of 'significant benefit' and 'added value' should be tackled. ECL believes that HTA bodies and payers should be involved in the design of the clinical trials, so that incentives can address disease areas with high unmet need. In defining unmet need, the voice of patients should be included in a wider platform coordinated by the Commission or an independent entity.

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 showed that regulatory flexibility can be effective to bring to the market new medicines for public health wellbeing and safety. Nevertheless, the rolling review should not be the rule. A communicable disease such as the SARS-CoV-2 requires immediate action but rare diseases, including rare cancers, do not require such a fast approach. There should be a balance between bringing new innovative medicines with proven clinical benefits and patient benefit.
- The fact that the Member States gave to the Commission the mandate to negotiate the price of COVID-19 vaccines is a great step towards a European Health Union. The same approach should be explored with the objective of reducing the price of expensive medicines.
- The COVID-19 pandemic outlined the importance of science but also the importance of people trusting in science. It is therefore critical to improve transparency with the contracts stipulated between the European Union/Member States and industry and the financial figures in these included.
- The COVID-19 pandemic outlined also the power of having the human capital, skills, knowledge, and advanced equipment to perform scientific studies. The Commission and the Member States could boost the intersect of the triple helix model of innovation with the cooperation of government with academia and private sector with a patient-centric approach. For instance, the Expert Panel on effective ways of investing in Health (EXPH) reports in its paper 'Innovative Payment Models For High-Cost Innovative Medicines' (2018) an example of the Triple Helix model to stimulate development of radiotherapy innovations led by the Karolinska university hospital in Sweden, together with other university hospitals, several private companies and government 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 type="radio"/>	<input checked="" 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 checked="" type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>

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

2000 character(s) maximum

When considering the eligibility for a medicine incidence criterion is better suited for rare cancers. See Casali, P., & Trama, A. (2019). Rare cancer agenda 2030. Ten recommendations from the EU Joint Action on Rare Cancers.

One-size-fit all incentive framework is not sustainable for the national healthcare systems, and it is important to differentiate with different levels of incentives/rewards and to simplify where possible. There is no need for additional incentives but rather to revise the system. The increasing number of new health technologies expected to come to the market in the next few years should not be equally incentivised. Considering their heterogeneity, existing incentives should be framed based (i) on the type of technology (the harder is the R&I process to develop a technology and the more it should be supported) upon evidence and clear criteria, and (ii) on the disease area (e.g., whether there are already treatments or not). The challenge is around the methods to differentiate the health technologies: those health technologies aimed at diagnosing, treating, curing the most neglected diseases should have the highest level of incentive (those diseases that are included in the 95%). Indeed, there is a concentration of medicines for certain areas while other disease areas are ignored. Finally, patient-reported outcomes on the treatment received should be taken into account when considering the criteria of unmet medical needs. (iii) If there are several medicines treating the same disease, the most beneficial one for the patient should be the threshold and rewarded while the others should see a reduction of the benefits. This approach might steer a patient-centric system for the development of treatments. HTA regulation with the Coordination Group and its alignment with experts (including patients) will be critical for this purpose.

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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The first important element is indeed having the possibility of conducting comparative clinical trials among health technologies. For this purpose, data and information sharing is critical. The European Health Data Space can play a major role in facilitating joint clinical assessments, but also with the post-marketing authorisation evaluations through the collection of real-world data and real-world evidence.

A product might tackle the disease, it might have an acceptable level of toxicity as well, but if the adverse effects are very intense the patient might decide to stop the treatment which would lose its value.

As a list of factors that can be useful to decide whether a new medicine has more benefits, we propose:

- Impact on survival;
- Level of physical and possibly mental adverse effects and the evaluation should come directly from the patients and specialist doctors;
- Impact on the quality of life communicated via patient-reported outcomes (e.g., how often the medicine needs to be taken, whether it is necessary to go to the hospital, impact of the treatment's effects on the ability to perform daily tasks, etc);
- The number of treatments the disease (both off-label and on-label use);
- The number of medicines with marketing authorisation specific for the disease.

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

All of the above. Yet, it is important to identify the root causes of unmet medical needs and determine whether, for example, the scientific knowledge of the disease is lacking, or whether the market is unattractive. It is important to avoid the multiplication of rare diseases.

Different root causes will need different solutions. For instance, medicines for children should require a different pathway. The 'First-in-Child' development and marketed authorisation of medicines against specific paediatric biological targets for the treatment of children with life-threatening and debilitating rare diseases, such as paediatric cancers, should be incentivized. In addition, reducing delays in starting the development of paediatric medicines and introducing tailored incentives should ensure the early start of paediatric cancer drugs development.

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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
Additional scientific support for the development of medicines from the European Medicines Agency	<input type="radio"/>	<input type="radio"/>	<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"/>

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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input checked="" 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

- Set an unambiguous and clear definition for the term 'unmet medical needs' (UMN), based on transparent and objective criteria. The definition should include but not be limited to the following: incidence, survival rates, existing alternative treatments, mortality and severity of the disease;
- The role of non-profit parties (i.e., academia and research institutes) in drug development should be enhanced and supported (e.g., access to technical assistance) to cover those disease areas with low commercial interest. They should have to access clinical trials' results and patient-level data including unpublished data from failed trials to enable high-quality assessments;
- Use alternative pull incentives that are publicly-funded, such as market entry or innovation rewards;
- Consider repurposing of existing drugs for treatments without viable markets. This can sometimes be done at low cost and can decrease both the time-frame and costs;
- Establishing mandatory involvement of HTA bodies, payers, and patients since the early stages of clinical trials' design ahead of the marketing authorisation request;
- Unveiling the potential of disease-registries by including treatment outcomes and patient-reported outcomes (PROs) and generously investing in the infrastructure of cancer registries. As the Staff Working Document (SWD) reports, 28% of all authorised orphan medicines did not need to demonstrate significant benefit because they targeted diseases for which there were no alternative treatment options. This means that patient-reported outcomes are essential for the evaluation of new medicines requesting marketing authorization;
- Ensuring that the collection and storage of data (incl. real-world data and real-world evidence as well as patient reported outcomes) fall within the remit of public authorities;
- Embedding the effective collection, storage, and analysis of PROs already at the clinical trials' design stage.

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

2000 character(s) maximum

It is relevant to reflect on the following sentence “Additional post-authorisation incentives that complement or replace the current incentives and rewards”. Before adding new incentives, we need to revise the current system and deal with inefficiencies. Indeed, for some OMP, the 10-year market exclusivity was not necessary to create a profitable product and the current incentive system indeed pushes toward the “salami-slicing” phenomenon.

Non-commercial entities, like academia, can play a vital role in developing potential medicines, especially in disease areas with weak commercial interest and where a low return on investment is likely. In the approaches listed in Q6, there is a strong focus on the development of the treatment, but the pipeline is already promising for limited disease areas (EFPIA pipeline review 2020) in terms of the number of new medicines (or combination of medicine and medical devices) coming to the market in the next years. Most likely, the most challenging barrier is the high prices that payers and national governments cannot afford whilst the number of patient diagnosed with a rare disease increases. Prioritisation will be needed and to do so it is critical to (i) employ cross-border horizon scanning initiatives to predict the costs and investments needed, (ii) boost transparency on the costs needed to place a product on the market, (iii) access to full clinical studies reports which would reassure the wider health community about the added value and safety of authorized products. Even before touching upon issues around payment and affordability, there is also the fact that not all the products are launched in all EU member states. This creates inequalities with regards the possibility of accessing the most advanced products that science would bring to patients. We look forward to the results of the pilot study on the market launch of centralised approved medicines.

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

All of the above, plus:

- Ensuring that the level of incentives is linked to the actual availability, accessibility, and affordability of pharmaceutical products across the EU.
- Considering unfair marketing behaviors with mergers between pharmaceutical companies and the consequent reduction of competition. DG competition, DG GROW antitrust unit for pharmaceutical market, and national antitrust authorities should continue to closely monitor the phenomenon and sanctions whenever it is needed. It is important to avoid monopolies within and outside Europe.
- The European Commission together with Member States should investigate and address the (marketing) tactic of withdrawing off-patent drugs and reintroducing similar medicines with new indications and higher prices [5]. The revision of the Directive 2001/83 will be critical in this regard.
- Discouraging practices which extend market exclusivity, prolong intellectual property protection, lead to competition distortion and profit maximization, through strategic use of intellectual property rights, such as incremental patenting of existing products (“ever-greening” strategies).
- Incentives should not lead to excessive profits as it happens for 14% of orphan medicines with an annual turnover above €100 million in the EEA (source, SWD).
- Boosting transparency and access to information about end-user prices, documentation of product value and the cost of developing and bringing the pharmaceutical product to market.

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

As the European Society for Paediatric Oncology (SIOP Europe) explains, the number of anti-cancer medicines available in child-friendly doses and formulations is far below the needs. Indeed, most oral chemotherapeutic medicines are not produced in child-friendly formulations and have to be compounded in pharmacies and pharmaceutical hospital departments.

Academic-driven research and development of child-friendly doses and formulations of essential anticancer medicines can play a pivotal role, particularly if supported by appropriate public funding. This approach can foster the production of financially accessible medications in a disease area where industry interest may be limited.

It should be noted that lack of child-friendly formulations is just one aspect of the multifaceted medicine access issues in the paediatric cancer sector. The lack of market-driven therapeutic innovation and shortages of essential medicines are contributing to stagnating cure rates and sub-optimal outcomes. As all paediatric cancers are rare and require cross-border cooperation to achieve progress, EU policies and programmes are ideally positioned to make a difference.

Please refer to SIOP Europe and CCI Europe (Childhood Cancer International – Europe) key recommendations for further details: <https://siope.eu/media/documents/recommendations-for-paediatric-cancer-following-launch-of-the-pharmaceutical-strategy-for-europe.pdf>

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

2000 character(s) maximum

As part of the EU Joint Action on Rare Cancers (2016-19), SIOPE Europe conducted a survey on the accessibility of essential medicines for paediatric malignancies ([https://www.annalsofoncology.org/article/S0923-7534\(20\)43223-5/fulltext](https://www.annalsofoncology.org/article/S0923-7534(20)43223-5/fulltext)). The results confirmed that academia/pharmaceutical departments in some countries are currently preparing ad hoc liquid formulations of several medicines for individual patients. At the European level, the European Society of Oncology Pharmacy (ESOP) Paediatric Working Group in liaison with SIOP Europe are running a project in this field and have good practices and recommendations to share.

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

2000 character(s) maximum

To ensure that developed products are reasonably profitable for companies and also reach patients, a fair pricing system should be in place.

A 'fair price' is justifiable, predictable and cost-effective within the priorities of the healthcare systems and the available budget. At the same time, a fair pricing policy that takes into account the ethical and financial dimensions of patient access to care, affordability and sustainability of healthcare systems should be encouraged and rewarded. Whereas 'justifiable' means a price that reflects the documented and clinically relevant benefit of the medicine, and a reasonable relationship between the cost of bringing the product to market (including R&D, production, marketing) and the price. For this purpose, it more transparency is needed. Whereas 'predictable' relates to the need for health payers, policy makers and systems to be able to predict the total costs and of investing in the treatment. 'Cost-effective' (ness) could be a common criterion for evaluating whether the price seems 'justifiable', as it links benefits with costs in a systematic way and provides a comparable decision-making tool across healthcare interventions. Finally, 'affordability' addresses the financial side of the sustainability of health systems. A prerequisite for obtaining fairer prices is a higher level of transparency and access to information about end-user prices, documentation of product value and the cost of developing and bringing the pharmaceutical product to market, as well as reimbursement decision-making processes. Policymakers should attach conditionalities to both national and EU public funding and ensure that public investment in R&D is accounted for and that medicines resulting from publicly funded research are available for a fair and affordable price. Furthermore, pricing and reimbursement authorities should be transparent about their decisions, how they are made, what criteria are used and who is involved in the process.

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