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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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European Federation of Pharmaceutical Industries and Associations (EFPIA)

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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

The innovative pharmaceutical industry investigates multiple areas of unmet need to develop treatment options for patients. The lack of therapeutic options in some areas is a consequence of existing scientific, regulatory and economic barriers to development and which are further compounded by uncertainties relating to Pricing & Reimbursement (P&R). Before taking any policy action, existing enablers and barriers to development need to be mapped and well understood to develop the right policies.

The OMP Regulation in 2000 introduced regulatory and economic incentives that have increased scientific activity and led to new treatments in the rare diseases (RD) space. These incentives have worked and should remain. The RD space is highly complex, covering more than 7000 varied diseases making it difficult for all of them to be addressed. The vast majority of RD affects only very few patients (89,1% of RD affect 11,4% of patients) which makes research both highly challenging scientifically and practically as well as, very often, unsustainable economically.

The Paediatric Regulation in 2007 introduced an obligation for all products developed for an adult population to also be developed for children unless there is strong justification not to. By design, it did not incentivise paediatric-focused development (i.e., without an adult reference population). Barriers to paediatric-focused development are scientific (i.e., lack of translational research), operational (difficulties to conduct trials in paediatric populations, preclinical data, recruitment and retention, trial design, informed consent) as well as practical and economic due to the small size and heterogeneity of the population that is further segmented into 5 different age categories. Furthermore, the industry has experienced challenges in conducting studies required by PDCO in PIPs.

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

COVID-19 has focused global attention on the pandemic, to the detriment of many other diseases, not the least in the field of rare and children's diseases. The effects are yet to be measured.

EFPIA members are proud to have contributed to the major scientific effort that led to preventative and therapeutic solutions against COVID-19 in a short time window. The technology (platforms) and science that resulted in vaccines and
therapeutics were already well understood and built on decades of research made possible thanks to the existing intellectual property framework.

During the pandemic, regulatory agencies have proven their agility, considering more global and rapid development, use and acceptance of preliminary data packages in rolling reviews and complementary /alternative evidence (e.g. RWE) as well as the use of conditional marketing authorisation, which could be valuable for OMPs and Paediatric medicines. Some labelling flexibilities applied to COVID-19 products could also be considered in the OMP/Paediatric product space to facilitate access across the EU. Sufficient resources must be ensured to allow this agility to continue.

COVID-19 vaccines and therapeutics have ongoing global paediatric development programmes based on EU and US paediatric investigational plans. EMA and FDA have increased collaboration during the pandemic and aim to agree as much as possible on a common strategy. In particular, all products aimed to treat or prevent COVID-19 were eligible for EU-US paediatric common commentary. This could be developed further for other medicinal products, to facilitate the development of paediatric medicines. However, it is clear that COVID-19 is anything but rare – other elements of the response (by industry, governments and others) to the global pandemic, potentially impacting the majority of the human population cannot directly be replicated or extrapolated to orphan and paediatric diseases that have very different characteristics.

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.	◉	○	○
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).

2000 character(s) maximum

The current orphan designation criteria are predictable and have been effective in encouraging the development of products for rare diseases. 98.2% of RD patients are affected by 10.9% of the more prevalent diseases (Wakap, et al., 2019). Lowering the prevalence criterion or introducing a cumulative prevalence criterion for products with more than one orphan designation would be a detriment to the majority of patients and will not redirect investment to rarer diseases. The proposal to add an incidence criterion for e.g. oncology products is concerning: rare cancers are rare diseases in their own right and it is very difficult to find relevant scientific literature to support findings on incidence. Incidence also discriminates against the deadliest diseases.

In order to address the concern around the relevance of orphan conditions, the definition of condition should evolve based on the scientific reality that conditions can be defined both by classic disease type /histology and by genetic disorders or deviations that cause disease.

The orphan designation criteria already establish that, where other treatments are available, only products bringing a clear benefit to patients should be incentivised (no available treatment or Significant Benefit, SB). This framework should be maintained and should remain different and separate from HTA standards.

All rare and paediatric diseases potentially constitute unmet needs, and the existence of treatment does not make a 'need met' per se. Additional incentives, such as transferable exclusivity extensions, could be considered for underserved areas characterised by a market failure. This should build on a thorough exercise of understanding the barriers to development. An analysis is being conducted by IRDiRC (Chrysalis project) – this and other projects should be considered by the EC before taking any policy action.

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?

2000 character(s) maximum

All factors considered in Commission notice 2016/C 424/03, clarifying the concept of Significant Benefit (Article 3(2) of Regulation (EC) No 847/2000) as 'a clinically relevant advantage or a major contribution to patient care' are important, including:

- Improved efficacy for all or parts of the population
- Better safety profile or tolerability for all or parts of the population
- Ease of self-administration
- Improved adherence to treatment.

Removing some of these elements would have a major detrimental impact on development of therapeutic options to address patients' needs. How these factors play into national health system decisions are within the remit of Member States and cannot be solved by EU legislation nor by using the regulatory tools designed for medicines authorisation.

Currently, COMP requests a late identification of the comparator, which makes it difficult to assemble meaningful data for the comparison. The SB framework would be improved if the cut-off point for the identification of the comparator therapy would be early in development (around phase II)to allow better evidence development and decision-making.

It should be clear that products used off-label, as well as compounded and hospital exemption products cannot be considered satisfactory treatments and should not be used as comparators for the purpose of the SB evaluation. In other words, only products with a marketing authorisation in the same indication should be considered for SB.

The procedure to confirm orphan designation criteria at the time of marketing authorisation should be streamlined, i.e., the COMP should start its review earlier in order to ensure that the designation confirmation is available and shared with CHMP ahead of the B/R evaluation. However, the CHMP B/R evaluation should remain clearly separate from the OD evaluation, including the SB assessment.

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

- [x] 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).
- [x] Treatments are already available, but their efficacy and/or safety is not optimal. For example, it addresses only symptoms.
- [x] Treatments are available, but impose an elevated burden for patients. For example, frequent visits to the hospital to have the medicine administered.
- [x] 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 constitute an unmet need, which is defined as a condition that is not adequately prevented, treated or diagnosed by authorised interventions. The incentives for development should not be restricted to a 'subtype' of unmet need. Restricting incentives to one or the other of the above propositions risks excluding the development of important therapies for patients.

As an example, in multiple myeloma (MM), treatment options prior to the early 2000s were limited, but the use of thalidomide as a treatment for MM in 1997, and its subsequent approval by the FDA in 2006, began an era of new drug approvals for myeloma including immunologic and other treatments. Following the rapid development of treatment options in the early 2000s, the primary challenge in the treatment of MM has shifted from limited treatment options to a challenge of identifying optimal combinations from the many options available. This would not have been possible if incentives had been restricted after the first treatment had been made available.

Since the introduction of the Paediatric Regulation, all programmes include the development of an age-appropriate formulation where relevant. However, approved formulations for some older products developed prior to the Paediatric Regulation might not be available. These products may instead be compounded from adult versions into a paediatric version in pharmacies or hospitals, even when newer treatment options with appropriate formulations might be available today. The current incentives have not attracted new development of paediatric formulations for older products, mainly because of the lack of willingness of healthcare systems to pay a higher price for a paediatric formulation when an older, cheaper adult version is available and can be compounded and used off-label. Novel incentives complementing existing ones, including national incentives to support uptake in the clinic, would support private investment in this area.

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	○	○	○	○	○	○	○	○	◉	○
Additional scientific support for the development of medicines from the European Medicines Agency	○	○	○	○	○	○	◉	○	○	○

Assistance with authorisation procedures, such as priority review of the application from the European Medicines Agency and/or expedited approval from the European Commission	○	○	○	○	○	○	○	◉	○	○
Additional post-authorisation incentives that complement or replace the current incentives and rewards	○	○	○	○	○	○	○	○	○	◉

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

2000 character(s) maximum

The innovative pharmaceutical industry is operating in a complex ecosystem often relying on a multitude of partners to bring an idea through to product development and ultimately, to patients.

For instance, research projects driven by academic centres or spin-off research centres affiliated with universities will work on an initial idea but will neither have the capacity nor capability to take on the high development risk and bring an asset to the market. Pharmaceutical companies will acquire the initial project (against a market-based price agreed between both parties).

A thorough analysis of the reasons for the lack of treatment in specific areas prioritised by society needs to be conducted. The international rare disease consortium IRDiRC is currently conducting a project (Chrysalis) which identifies key criteria that would make rare diseases research more attractive to the industry for research and development (https://irdirc.org/activities/task-forces/).

From efpia perspective, the following factors are important in deciding whether to invest in a disease area and/or targeted molecule:
- Scientific/clinical factors (e.g., disease knowledge, infrastructure, company expertise)
- Economic factors (e.g., size of patient population, competitive landscape, time to market)
- Policy factors (e.g., IP incentives, regulatory, P&R frameworks)

Where the main barriers are scientific, i.e., where a disease aetiology and pathophysiology are not sufficiently understood or where no targets are identified/available to treat the disease, partnership and multi-party collaboration could support the progress of research activities.

Where the main barrier is an economic one e.g., an extremely small patient number, novel incentives such as transferable exclusivity extensions could improve the economic viability of medicines development. Market-level incentives are also critical, with the value of the medicine being appropriately recognised by healthcare systems.

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

2000 character(s) maximum

Regulatory support has been and continues to be a very useful mechanism. Protocol assistance for the development of OMPs is highly valued. However, if additional responsibilities are put on the regulatory framework and EMA, relevant resources also need to be provided. To tackle COVID-19, the regulatory framework has had to reallocate substantial resources, and this is not sustainable in the long run. More resources and better integration of all development support activities should be considered for a scientifically strong regulatory system in Europe. In this way Europe's global influence could also be fostered through stronger scientific alignment on a global level through bilateral and multilateral initiatives and collaborations. Any further divergence between jurisdictions should be avoided because additional hurdles for global development plans would be detrimental.

It is not fully clear what is meant by 'post-authorisation incentives.' If novel incentives such as transferable exclusivity extensions would be considered, there must be a thorough analysis of the landscape to identify which areas should be eligible for such measures and under what conditions, e.g., in terms of transparency of the extension granted, timelines to be followed, or ability to combine any novel incentive with other existing support measures. Furthermore, any new post-authorisation incentives should be complementary to current incentives and not replace incentives that have already been proved to be effective.

Any policy measure providing incentives must include the right safeguards to ensure that there is no negative impact on the development chain.

Q7: Which of the following options, in your view, could help <u>all</u> 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

The solutions proposed are ill-conceived and might be counterproductive as they do not take into consideration the spectrum of drivers that effectively impact patient access. Rare and paediatric diseases are not attractive commercially which is why there has been little generic penetration thus far. Constraining the commercial freedom of companies (by e.g., linking incentives to launching in markets, where market launch is not only dependent on a company decision) will not positively impact patient access (given the strong correlation between access and reimbursement) and will act as a negative signal for conducting research in Europe, therefore making Europe less attractive than it is today. Companies today already engage in licensing deals and transfer their products to another company when there is shared interest on both sides. Nothing needs to change to allow this to happen.

Access is dependent on a number of mostly national and health system related factors and market dynamics that cannot be foreseen at the time of R&D investments. These include a complex interplay of factors, out of which many are outside of a company's control. EFPIA has carried out significant work on the multi-factorial reasons behind the unavailability of medicines and delays and is currently initiating a timely collection of the considerations underlying unavailability of centrally approved products and the degree to which this reflects: i) barriers within the environment, and ii) commercial decisions arising in light of Member States' P&R processes.

Hurdles to patient access to innovation cannot be solved through the review of the Paediatric and Orphan Regulations and need to be tackled through a careful analysis of root causes of access delays and lack of availability of specific products in specific markets.

It is vital to bring all stakeholders around the table. We support setting up a High-Level Forum on Access to Innovation to co-create solutions.

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

In order to guarantee children's safety, it is particularly important to ensure that the medicinal products that are prescribed follow the appropriate dosage and formulation suitable for use in younger patients. Children are not 'smaller' adults but present specific biological and physiological differences which need to be properly accounted for. A paediatric formulation of an older existing product will need to meet the same quality, safety and efficacy requirements as any other medicinal product, but a sponsor will only undertake such a development if relevant incentives for the necessary investment are in place.

EFPIA companies develop innovative products to translate cutting-edge science into new treatment options for patients, including paediatric formulations for any new compound if appropriate. In accordance with the obligations of the Regulation, many paediatric formulation developments have been performed for older products that are still patented.

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 or philanthropic funding to support SMEs, academia, or contract manufacturers may be needed to provide the necessary stimulus for the development of paediatric formulations of off-patent products, taking into account the challenges of producing small volume products such as paediatric formulations.

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

2000 character(s) maximum

It must be ensured that products are prescribed and reimbursed, recognising their value. If there are no complementary national push incentives and willingness to pay for additional new formulations by the 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.

Contact

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