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Feedback from: Association of European Cancer Leagues (ECL)

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Medicines for children & rare diseases – updated rules *(/info/law/better-regulation/have-your-say/initiatives/12767-Medicines-for-children-&-rare-diseases-updated-rules_en)*

The ECL Access to Medicines Task Force (A2M TF) welcomes the Commission's initiative to seek a new legal framework to solve issues within current legislation on medicines for children and rare diseases. The A2M TF urges the Commission to prioritise the following problems:

There are no approved treatments for 95% of rare diseases. This must change. Therefore, the A2M TF strongly agrees with the Commission that medicines should be developed in areas of unmet medical needs. To encourage real innovation in areas with high unmet medical need, public and private investment in R&D should be steered towards clearly defined public health needs. This should include medicines for children and rare diseases. To achieve this, an improved coordination and priority-setting mechanism is needed at the EU and global level. The new legislative framework should provide incentives to stimulate developments for patients with so-called ultra-rare diseases and for children's most pressing needs, such as medicines for children with rare diseases, with extra attention for children with cancer. For children, the 'mechanism of action principle' should be introduced. This prevents granting of PIP waivers when an adult cancer has no paediatric iteration but the drug's mechanism of action is beneficial for pediatric cancers.

The incentives must reasonably reward the level of novelty of a product. The regulation should tackle threats to affordability and access, such as incentives leading to overcompensation, indication stacking, and high drug prices even when the R&D investment of a company was very low. At the same time, it should deter inappropriate use for so-called "artificially rare diseases" and multiplication of exclusivities in all forms.

The new regulation should introduce experiments with innovative incentives. An example is a system based on rewards or prizes. The reward compensates for the R&D cost, so that the drug can be distributed in every European country at the cost of production and distribution.

The EU faces huge inequity challenges. There exists too big of a difference between and within Member States when it comes to access to medicines. In some Member States, certain treatments are delayed or do no enter the market at all. Children and patients with rare diseases must have equitable access to affordable treatment. It should not matter where you live.

The A2M TF sees a problem with the lack of legal conditions connected to incentives (e.g. SPC's) that could enhance wide market availability. EU wide access could be guaranteed by introducing an obligation for providers to introduce new orphan treatments in the entire European market. Dedicated funding to support development of orphan drugs by SMEs and academia should come with conditions leading to affordability. "Socially responsible licensing" is a guiding framework for these conditions. Transparency is fundamental. The fact that the Commission cannot reassess a provided designation on its effect for availability, affordability and accessibility might lead to problems connected to misuse of the regulation. To conclude: it lacks an intervening tool that keeps the paediatric and orphan pipeline healthy.

In addition, academia plays a vital role in developing treatments in the area of unmet medical need. The new legislative framework must value their efforts and ensure a level playing field for academic sponsors. Therefore, the regulations must extra support academic sponsors. To ensure that the pool of non -commercial sponsors enlarges, and thereby more new treatments for children and patients with rare diseases are developed.

The ECL A2M TF wishes to contribute to this stakeholder wide discussion on the matters as described above. If it would be the Commission's preference, the ECL A2M TF welcomes any further dialogue and endorses the position submitted by the European Fair Pricing Network (EFPN). Please see p.15-17 of the attached position paper on the EU Pharma Strategy.

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