Revision of the EU legislation on medicines for children and rare diseases

EuropaBio Inception Impact Assessment Response

Developing medicines for children and rare diseases is a lengthy and complex undertaking. Much less is known about these illnesses when compared to more common conditions, meaning extensive research is required before scientists and researchers can seek to transform their ideas into potential treatments.

Funding to conduct such research is vital for biotech companies, especially SMEs, to bring their investigational therapies to life. These companies need to make a very strong case to convince potential investors to incur the high economic risk of early-stage research and lengthy therapeutic development timelines, particularly when the number of patients is limited, and the condition is difficult to characterise. The study to support the evaluation of the EU Orphan Regulation reveals that 86% of turnover levels for orphan medicines are below €100m in Europe with an average annual turnover (2008-2016) of €56m, showing that financial barriers to development still exist.

Since 2000, the number of medicines in development for rare conditions has increased consistently with over 2000 medicines for rare diseases in development by the end of 2020. Regarding the development of paediatric medicines, the European Commission's 10-year report found that between 2007–2016, over 260 new medicines for use by children were authorised.

For these reasons, EuropaBio believes that any evaluation of the EU legislation on medicines for children and rare diseases should have, at its heart, the goal to build on the current success of the existing legislation and to encourage further investment in rare diseases in order to increase and accelerate innovation.

Unmet medical need should not exclude areas where treatment is available but encompass areas where patients still need better outcomes. For paediatric indications, it is unrealistic to assume that only rewarding research in unmet need would attract more R&D effort. On the contrary, the evaluation as proposed could lead to more obligations for limited rewards.

Lowering prevalence criteria, eroding market exclusivity, limiting access to the Orphan Designation, or reducing the possibility to develop medicines in multiple indications, will complicate biotech companies' efforts to attract long term investments. It will only increase the attractivity of other regions in the world for R&D. Similarly, linking rewards to market launch requirements will be counterproductive. Instead, the EC should fully focus on potentially viable incentives for encouraging further research and making the EU a future leading region in the development of medicines for children and rare conditions. Extending the EMA's PRIME scheme to orphans, increasing the access and dialogue to EMA early advices in particular for SMEs, introducing additional regulatory flexibility or developing public-private partnerships targeting

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areas of specific relevance could be explored. In general, the orphan market is small, it is only innovation, and not biosimilars/generics, that will create the treatments needed in the future. EuropaBio understands there are concerns regarding the differences in availability of certain medicines among the EU Member States. These concerns are often the result of fragmented data requirements, reimbursement processes, and bureaucratic timelines, which fall mostly within the national competence. These concerns can, and must, be addressed without jeopardising EU legislation that successfully encourages research into these conditions of unmet need. **Trying to address these access issues through a weakening of a carefully crafted European incentives system could ultimately have the opposite effect to the objective sought for.**

In conclusion, EuropaBio encourages a discussion with all stakeholders, comprised of public officials, patients, regulators, payers, and industry, to address the fragmented access pathways for innovative therapies among the EU Member States and discuss how processes could be streamlined or accelerated.

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About EuropaBio

EuropaBio, the European Association for Bioindustries, promotes an innovative and dynamic European biotechnology industry. EuropaBio and its members are committed to the socially responsible use of biotechnology to improve quality of life, to prevent, diagnose, treat and cure diseases, to improve the quality and quantity of food and feedstuffs and to move towards a biobased and zero-waste economy. EuropaBio represents 75 corporate members and 17 national biotechnology associations and bioregions.

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