EuropaBio response to the European Commission Public Consultation on the Regulations for Orphan Medicinal Products and Paediatrics

PRESS RELEASE

Brussels, 2 August 2021 - EuropaBio has responded to the European Commission's public consultation on the Orphan Medicinal Products (OMPs) and Paediatrics Regulations.

EuropaBio made it clear that the EU incentives framework has successfully driven the development of medicines for rare and paediatric conditions, with the number of medicines in development significantly increasing since the introduction of the legislation (with 3,678 applications for orphan designation submitted by the end of 2020[1].)

It is estimated that 80% of rare disease patients suffer from just 4% of the known rare conditions[2]. While most orphan drugs developed so far address conditions affecting these 80% of patients, this still leaves a significant number of patients lacking sufficient treatment options, particularly those with conditions which can be considered "ultra-rare."

EuropaBio acknowledges the need to further encourage R&D in these under-served conditions and is ready to engage all stakeholders to search for potential solutions. To begin this discussion, in its response to the consultation, EuropaBio has suggested several options worth exploring e.g., a new model for public-private partnerships, building upon the European Reference networks, and utilising the upcoming European Health Data Space for rare disease research.

EuropaBio emphasises the immensely positive impact the incentives framework has had on encouraging the development of new therapies for rare and paediatric patient groups. We therefore call upon the European Commission to approach any proposed revision of the legislation with utmost caution, carefully avoiding any potentially negative impact on the environment for rare & paediatric R&D in the EU. Any revision of the legislation should learn from, and build upon, its prior successes, while paying careful attention not to undo them.

EuropaBio remains fully committed to being a constructive partner in the ongoing and upcoming discussions, in the interest of encouraging the development and delivery of new therapies for patients.

To view our full response to the public consultation, please click here.

[1] European Medicines Agency, *Annual report on the use of the special contribution for orphan medicinal products 2020* https://www.ema.europa.eu/en/documents/report/annual-report-use-special-contribution-orphan-medicinal-products-2020_en.pdf

[2] Nguengang Wakap, S., Lambert, D.M., Olry, A. et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet 28, 165–173 (2020)

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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 corporate and associate members across sectors, plus national and regional biotechnology associations which, in turn, represent over 1800 biotech SMEs. Read more about our work at www.europabio.org.