

Statement by EuropaBio in response to the publication of the European Commission's evaluation of the Orphan and Paediatric Regulations

13 August 2020

EuropaBio notes the recent publication of two key documents by the European Commission affecting the rare disease and paediatric patient communities, notably the [European Commission Staff Working Document](#) evaluating the Orphan and Paediatric Regulations, as well as the accompanying [study to support the evaluation of the EU Orphan Regulation](#) to which EuropaBio participated.

EuropaBio welcomes the major findings of the reports that, since their introduction, both the Orphan Medicinal Products and Paediatric Regulations have fostered the development of new orphan and paediatric medicines, improved the overall EU climate for Research & Development, and provided for faster and increased access to innovative treatments in the EU.

As the association representing the European biotechnology industry, supported by our network of associations representing the sector at national level within the EU/EEA, EuropaBio was pleased to note the reports found that 53% of sponsors for orphan medicinal products are headquartered in the EU, and that 40% of all orphan designations are held by SMEs. EuropaBio feels it is imperative that the success and momentum catalysed by these Regulations is not lost, and that the EU ensures it can remain a hub for cutting-edge medical research, particularly for the rare disease and paediatric communities.

We note with concern that, despite the remarkable increase in the development and availability of new, innovative therapies for these communities, some inequalities in access continue to exist within the EU. We concur with the findings of the Technopolis/Ecorys study, supporting the Commission's evaluation, that this problem largely lies outside the scope of the Regulations, and that *"a substantial part of the unevenness stems from national policies and decision-making processes"*. EuropaBio stands ready to work with the European Commission, and all other interested parties, both public and private, to identify and propose innovative solutions to access inequalities within the EU. However, we emphasise that the important contribution of these Regulations to the development of such therapies should not be overlooked.



An opening of the Orphan Medicinal Products Regulation to address issues outside its scope is a risky undertaking. As stated by the Technopolis/Ecorys report: “any modifications to the regulatory framework, particularly to the instrument of market exclusivity, solely for the purpose of better bringing in line costs and rewards could have the undesirable ‘side-effect’ of also slowing down much needed innovation”. We thus note with concern that, despite the positive findings of the Technopolis/Ecorys report, the Commission indicates that the Regulation requires legislative change, an approach we strongly advise against.

EuropaBio believes that restoring Europe’s rightful position as a global leader in healthcare biotechnology and biopharmaceutical research should be a leading objective of the EU’s pharmaceutical strategy. We remain available for dialogue with both EU and national institutions to discuss how the common aim of incentivising research and increasing patient access to the resulting therapies of that research can be achieved.

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Media contact

Kai Künnecke, EuropaBio Communications Officer

Email: k.kuennecke@europabio.org/ Telephone: +32 2 739 11 81

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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