EuropaBio response to the European Commission Public Consultation on the Regulation for Orphan Medicinal Products and Paediatrics: the value brought by the technology to patients and society should be well recognised

Explanation of EuropaBio's response to Question 8

PRESS RELEASE

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EuropaBio responded to the European Commission's (hereafter, EC) Public consultation on Medicines for children & rare diseases. As the EU rules to incentivise the development of medicines for children and for people with rare diseases have been in place for nearly 20 years, the EC aims to ensure that products addressing the specific needs of children and patients with rare diseases are developed, the groups have timely access to medicines and there are efficient assessment & authorisation procedures.

This document serves to clarify EuropaBio's response to a specific question contained within the European Commission's public consultation on the revision of EU rules for medicines for rare diseases and children. In the absence of the opportunity to clarify our answer within the questionnaire, EuropaBio would like to provide an explanation in written format.

Question 8 invites respondents to answer the following question: *"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?"*

The question is then followed by three possible answers:

1. Both new, innovative medicines and well-known medicines repurposed to treat a rare disease should receive the same reward
2. New, innovative medicines to treat a rare disease should receive an enhanced reward
3. Do not know/cannot answer

EuropaBio has chosen to respond to question with option number 1, namely that **both new innovative medicines and well-known medicines repurposed to treat a rare disease should receive the same reward.**

EuropaBio, and its members, are strong advocates of encouraging a positive environment for breakthrough innovation. However, we believe medicines that have been repurposed in other disease areas or indications for new medical uses can also have tremendous value. We feel it is not the technology itself that should be rewarded, but rather, the value it brings to patients and society that should be recognised.

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In the Regulation on Orphan Medicinal Products (EC 141/2000), the legislation states that an orphan designation can be granted when the product is *"intended for the diagnosis, prevention, or treatment of a life-threatening condition, seriously debilitating or serious and chronic condition in the Community and that without incentives, it is* ***unlikely that the marketing of the medicinal product in the Community would generate sufficient return to justify the necessary development."*** Such incentives are key in encouraging investment in repurposing products for rare indications, which may not be economically viable under other circumstances without incentive.

Furthermore, orphan designations are granted only when there is insufficient treatment for a particular condition, or when the product is safer, more effective, or clinically superior when compared to existing treatments. A potential medicine does not need to be a breakthrough innovation to greatly improve the quality of life and management of a condition. Encouraging sponsors to explore existing medicines as potential therapies for other conditions with unmet medical need should continue to be encouraged as, in some cases, a repurposed product may also present more value to a rare disease community than a new molecular entity. It is, therefore, the value of the product, not how it was developed, that should be rewarded.

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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, plus national biotechnology associations and bioregions.

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