



THE REVISION OF THE OMP REGULATION - PERSPECTIVES FROM THE RARE DISEASE COMMUNITY

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Five overarching principles for European leadership

1

Evolve incentives framework whilst maintain predictability for sponsors, enhance Europe's competitiveness, benchmark with US competitiveness

2

Drive investments in underserved areas whilst maintain incremental research in diseases with some treatments.

3

Transform European R&D ecosystem for RD building upon advances of the past 20 years

4

Keep up with the scientific development related to transformative medicines

5

European pathway from development to access, as an incentive for sponsors to ensure innovation coupled with affordability

Several levers to achieve this ambition including the revision of the OMP legislation which is a key cornerstone, as well as the strengthening of EU clinical research capacities

Strengthen EU clinical research capacities - ACT-EU (Accelerating Clinical Trials in the EU)

Infrastructures / existing networks

- ERNs → clinical research capacities (CSA ERICA)
- EJP-RD → RD partnership with Clinical Research Networks
- Together4RD → public private partnership in clinical research

Projects / infrastructures in- building

- Conect4children → Paediatric Clinical Research Networks
- REMEDI4ALL → Platform for repurposing approaches

Counterparts at global level

- IRDiRC, RDI & Global Network for RD
- Bespoke Consortium and new IHI call on ATMPs

Concrete proposals for a responsible evolution of the RD incentives framework: to lead and show a new way



Maintain the **prevalence threshold** to leave no disease behind



Address **unmet needs** through structured multi-stakeholder early dialogue



Guide the development of treatments with an Orphan Drug Development Plan



Modulate the incentives including market exclusivity towards clear unmet needs



Introduce the new concepts of **conditional significant benefit**



Strengthen the remit of the Committee for Orphan Medicinal Products (COMP)



Include the **PRIME** scheme as an incentive + new ones such as **EU as 1st market**