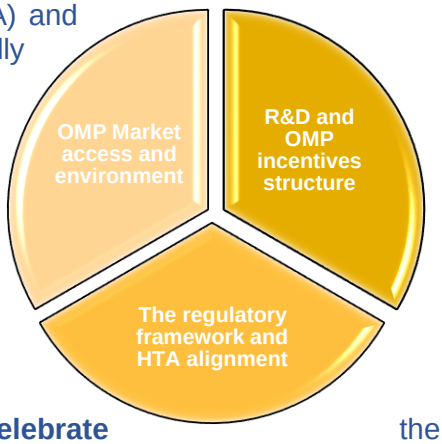
EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

"The Orphan Medicines Regulation - the Way Forward"

The European Commission launched an evaluation of the Orphan Medicinal Product (OMP) and Paediatric Regulations. EUCOPE seizes the opportunity to contribute to this process, outlining **ways to strengthen the EU environment for the development of OMPs.** The elements are the OMP Regulation itself, the broader pharmaceutical regulatory framework, Health Technology Assessment (HTA) and national market structures for access to OMPs. These elements *should* be fully complementary and integrated, **however, gaps still remain:**



OUR VISION

Bridging the gaps in the EU environment for the development of rare disease treatments with actionable proposals that **strengthen the cycle of biopharmaceutical innovation for rare disease patients.**

GAPS IN THE RARE DISEASE ENVIRONMENT

The **20th Anniversary** of the **OMP Regulation has been an opportunity to celebrate** the substantial achievements of the EU in fostering research to the benefit of rare disease patients, their families and carers.

20 YEARS OF OMP REGULATION

- *From 8 to 182 OMPs*
- *Benefitted 6.3 million patients in the EU*
- *Support the establishment and growth of OMP focused companies and SMEs*
- *48% of OMPs have revenues of less than €10m in the EU*
- *The overall cost of OMPs is estimated at around 7% of pharmaceutical spending in Europe(approx. €10.5bn per year)*

There are however more than 6,000 rare diseases and **95% of these still do not have an authorised treatment option**. Patients are unevenly distributed across these 95%: **400 of the 6,000 rare diseases account for 98% of rare disease patients**, while only 149 of the 6,000 rare diseases account for 77-81% of patients. **For the 5%** of diseases that do have a treatment, the patient journey is far from simple.[1]

In disease areas with few patients globally, there are enormous issues related to scattered and scarce data, which negatively impact on R&D and regulatory approvals, and delay patient access. **Evidential uncertainty and differing requirements along the lifecycle of OMPs, from development to launch,** pose significant hurdles, especially to smaller innovative companies heavily focused on treatments for rare diseases.

The Commission evaluation of the OMP Regulation highlights that **OMPs are not equally accessible to patients** in all EU countries. Access to medicines is primarily a Member State competence. Member States policies, plans and strategies for rare diseases vary greatly across the EU. Indeed, **only some countries have distinct processes for OMPs** in place[2] and **delay in patient access to OMP across Member States can vary from few months to years**.

[1] EURORDIS Rare Barometer: 7500 respondents: 69% of rare disease patients had received treatment for their rare disease, only 5% had received a transformative treatment approved for the entire European Union

[2] For instance, in England, Scotland, and Germany

OUR COMMITMENT: EUCOPE leads and partakes in targeted partnerships aimed at developing actionable proposals to strengthen the **EU environment for the development of OMPs**.

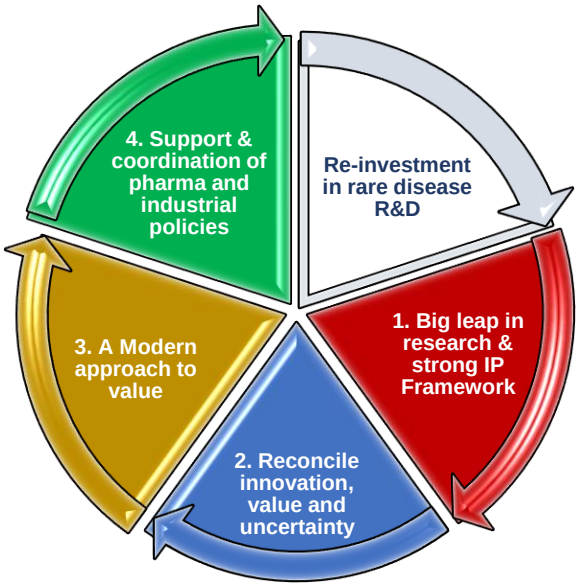
EU environment for the development of OMPs

Innovation	R&D	(Post) marketing authorisation & P&R
The European Expert Group on Orphan Drug Incentives Multidisciplinary expert group including researchers, academia, patient representatives, investors and industry Input to strengthen the OMP Incentives Environment in Europe focusing on **addressing unmet needs** and supporting delivery of continued innovation where treatments exist; finding ways to optimise and accelerate **OD regulatory pathways**; and identifying actions tailored to support **OD development** and industry and academia collaboration.	Guidance on **defining uncertainties and evidence gaps in the assessment** of value of treatments for rare diseases and **better leverage the potential of RWE for research**	**Approach to RWE support to Payers/HTA**: the partnership seeks to establish a learning network. Recommendations on **adopting standards and legal basis for use of Real-World Evidence**
European Expert Group on OD Incentives	RWE4Decisions REAL WORLD EVIDENCE TRUST4RD	RPH-VAL

OUR RECOMMENDATIONS

1. *Develop more incentives for biopharmaceutical companies active in rare diseases to overcome challenges in basic research and clinical development. A **big leap in funding of new research** is very much needed to better address unmet medical needs and identify new solutions.*

2. *Optimise and streamline regulatory pathways for OMPs **to reconcile innovation value and uncertainty**. This includes more acceptance of **Real-World Evidence (RWE), closer European collaboration on HTA** and further alignment between regulators and payers' requirements.*



3. *Ensure consistent and widespread support across Member States to develop and implement the **HTA Value Frameworks (VFs)**, which capture the broader benefits the OMPs bring to patients and their families, health systems, the economy, and society. This will require **a modern approach to value and the uptake of innovative payment models.***

4. ***OMP policies need to be supported and coordinated with pharmaceutical and industrial policies at** national and EU level.*

This virtuous cycle allows for **re-investment in R&D research of innovative therapies for rare diseases patients.**