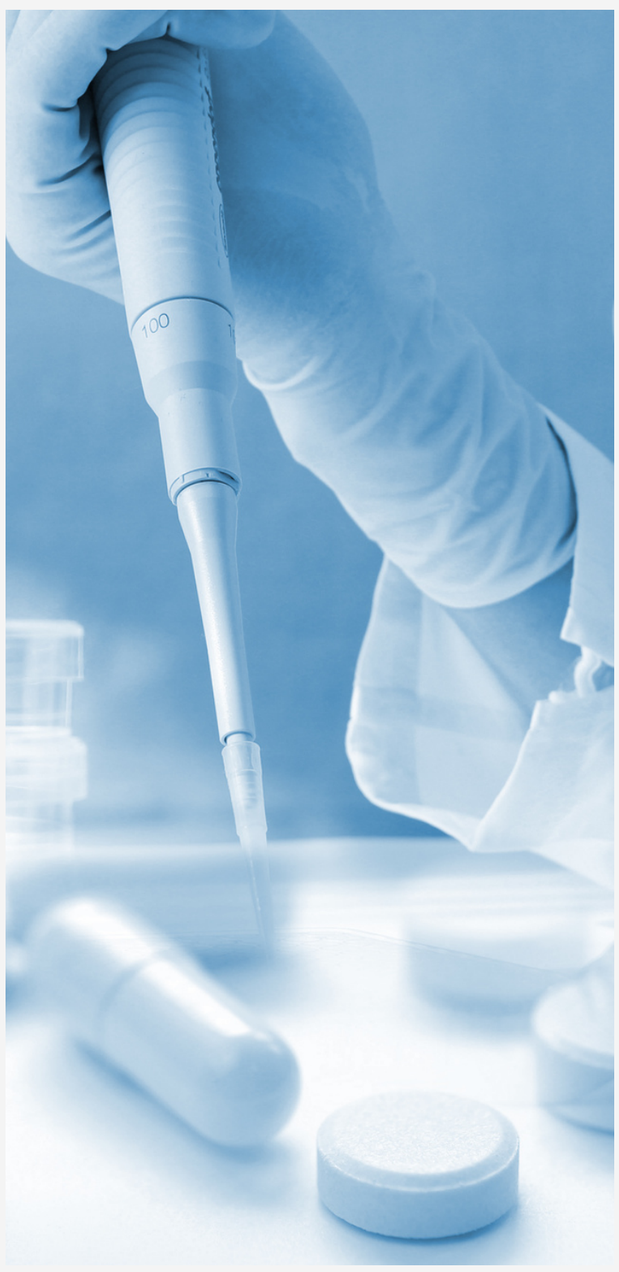
EUCOPE

European Confederation of Pharmaceutical Entrepreneurs AISBL

POLICY BRIEF

Orphan Medicinal Products (OMP) Regulation



ABOUT EUCOPE

The European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) is the voice of small to mid-sized health technology companies in Europe. Representing 2600+ innovative biopharmaceutical companies directly or through national associations, EUCOPE advocates for sound public policy that supports innovation, while fostering a community built on a shared purpose: improving and saving the lives of European patients through innovative therapies and medical technology.

The European Commission launched an evaluation of the legislation on medicines for rare diseases and children. EUCOPE seizes the opportunity to outline ways to **strengthen the whole EU environment for the development of rare diseases treatments**, of which the Regulation (EC) No 141/2000 on orphan medicinal products (OMP Regulation) is a key pillar.

Learn more about the OMP Regulation and the revision process

Our Approach and Commitment

The new OMP Regulation should build on the success of the current system and recognise that unmet needs in rare diseases cannot be addressed by means of this revision alone. Thus, EUCOPE looks at solutions along the **whole lifecycle of rare disease therapies through multi-stakeholder collaboration**, involving payers, medical and research community, biopharmaceutical industry and patients among others. Examples of this approach are the European Expert Group on Orphan Drugs Incentives and RWE4Decisions. EUCOPE's membership consists of European and global companies committed to ensuring that **Europe remains an attractive location to undertake research and launch products for people living with rare diseases.**

Our Proposals for Commission, Parliament and Council of the EU

Support an environment that fosters rare disease innovation in Europe: the EU needs to strengthen its OMP R&D ecosystem by focusing on supporting the pre-clinical and clinical research into OMPs, building on existing initiatives such as the European Joint Programme on Rare Diseases, the European Reference Networks (ERNs) and the emerging European Health Data Space.

Create a broad rare disease unmet need framework that attracts developers to underserved areas: rather than defining unmet need in the OMP Regulation, we call for a broad, *criteria-based approach* to orphan drug designation (ODD) and incentives that goes beyond the absence of any approved therapeutic option. To ensure predictability and stability of the system, the *current prevalence threshold (5 in 10000 people)* should remain the main ODD criterion

Increase stability and predictability of the OMP regulatory system and alignment of evidentiary requirements across the medicine lifecycle: a vital precondition to ensure continued investment in OMP R&D. The concept of *Significant Benefit* plays a pivotal role in this respect and should remain a corner stone of the OMP Regulation and serve as a building block for P&R processes at Member States level.

Develop a thoughtfully calibrated incentive design: modulating market exclusivity alone will not suffice, especially if it only consists of a reduction of the current exclusivity period. While *market exclusivity should remain the main tool of the OMP Regulation*, additional rewards need to be carefully designed to incentivise OMP developers to go into *areas where standard innovation models alone might not be effective.*

Read the EUCOPE Position Paper on the OMP Regulation