A 20th Anniversary Analysis of the EU OMP Regulation: Achievements & Future Outlooks

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Introduction & Rationale for our Study

In view of the 20th anniversary of the EU OMP Regulation, EUCOPE commissioned a **study from the UK Office of Health Economics** (OHE), aimed at:

· **Assessing how far the Regulation has incentivised developers** to invest in the R&D of OMPs;

· **Analysing the economic and financial challenges of developing OMPs** and measuring impacts of changing core legislative elements;

· **Analysing the ongoing competition** in the market of OMPs.

EUCOPE framed the analysis in the political context to contribute to the ongoing debate on the EU OMP Regulation evaluation and lay the foundations for future policy recommendations on OMP incentives.

The economic and financial challenges of developing OMPs

· **OMPs generate lower and more volatile returns** than medicinal products for common diseases, due to **high failure rates, barriers for patient access and real-world data generation** challenges;

· **Based on a Net Present Value Model**, our analysis shows that **changes to the Regulation would lead to a decrease in the number of OMP developers** and, in some cases, **would threaten the financial sustainability** of OMP-focused companies.

The value of OMPs beyond healthcare budgets

There are numerous areas of value OMPs bring unaccounted for by HTA metrics.

· The annual cost of supporting a patient with Duchenne muscular dystrophy exceeds €100,000, with similar implications for family caregivers;

· 34% of parents of children with cystic fibrosis reported clinical depression;

· **OMPs can contribute to quality of life and well-being of carers and family members, which can boost productivity and offset OMP costs.**

The Regulation's impact on OMPs R&D

The Regulation has successfully incentivised companies to invest in the development of OMPs:

· Marketing authorisations (MA) granted to orphan drugs between 2000 and 2018 grew at a **compound annual growth rate of 15%**;
· More than a **third (35%)** of granted MAs are held by OMP-focused developers.

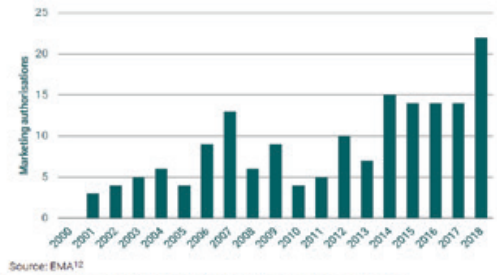

Figure 1: MARKETING AUTHORISATIONS OF OMP PER YEAR 2000-2018

Competition in the OMP space

The EU OMP Regulation does not create monopolies:

· Nearly a sixth of all OMP indications were treatable by more than one product still protected by market exclusivity; More than **one out of six OMPs with expired market exclusivity faced competition** from at least one generic version;

· The relative lack of overt competition is **a feature of OMPs due to the small populations** and the rare incidence of the conditions they target **rather than the longer market exclusivity period**;

· **In the mid-term, competition is likely to increase** as the exclusivity of products that were approved over the past decade expires in the next 3-5 years.

Key takeaways and Conclusions

· **Maintaining a strong incentives framework for OMPs is vital** for Europe to thrive as a major innovation hub for rare diseases;

· Remarkable results have been achieved thanks to scientific breakthroughs and an incentive ecosystem. **However 95% of rare disease patients still do not have any treatment option**;

· **What's next**: To address unmet needs, policymakers will have to take into account:
  · The **specific financial challenges of OMP development**;
  · How **OMP development contributes to the innovation ecosystem** in Europe;
  · **Patient-relevant and society-relevant unaccounted value** of OMPs.

Reference: Berdud, M, Garau, M., O'Neill, P., Rozanova, O., Bell, E. (2020). Economic and Financial Challenges of Developing Orphan Medicinal Products. Does the European Regulation Tackle them?, OHE Consulting Report, London: Office of Health Economics.