ORPHAN MEDICINE INCENTIVES

How to address the unmet needs of patients by transforming the European OMP landscape

European Expert Group on Orphan Drug Incentives
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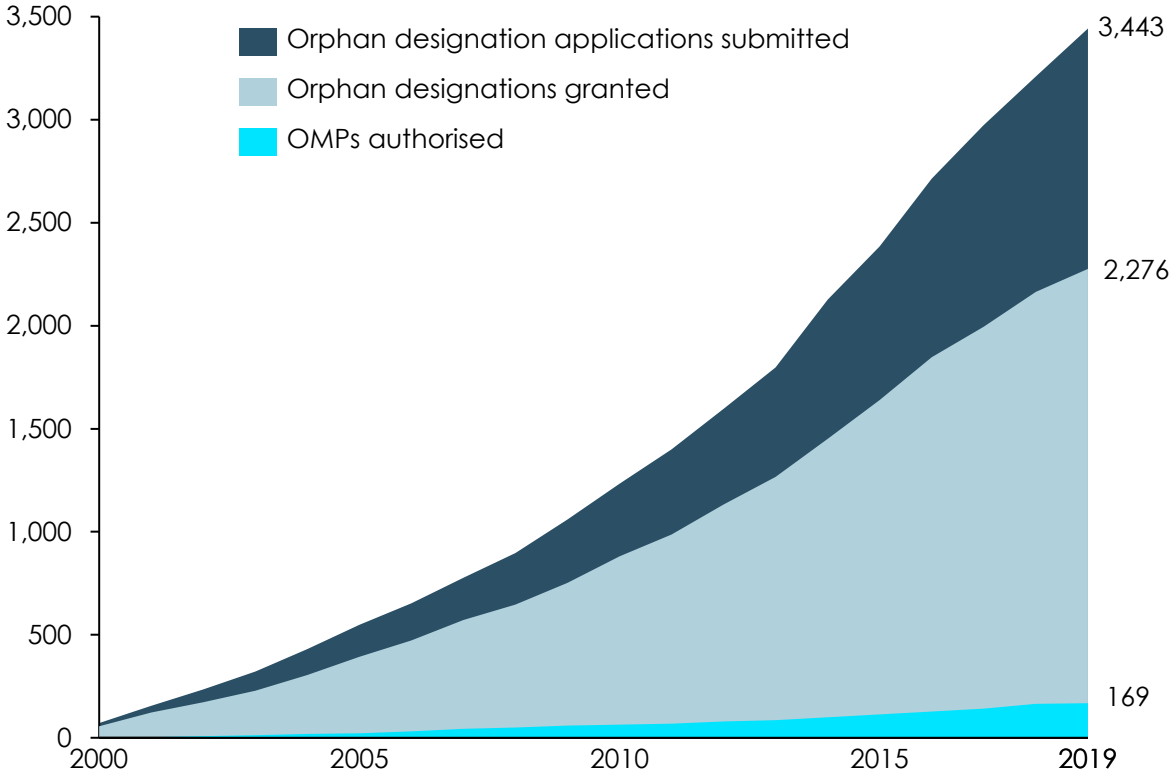
1
A LOOK BACK

2
THE PRINCIPLES

3
THE POLICY IMPROVEMENTS

A look back: the OMP Regulation has been a success but there is still unmet need

Applications submitted, designations granted and authorised OMPs since 2000
Cumulative



OMPs available for rare diseases
Share of all rare diseases

Source: European Commission (2020), European Medicines Agency (2020), Wakap et al. (2020)

Four guiding principles for the revision of the OMP policy framework

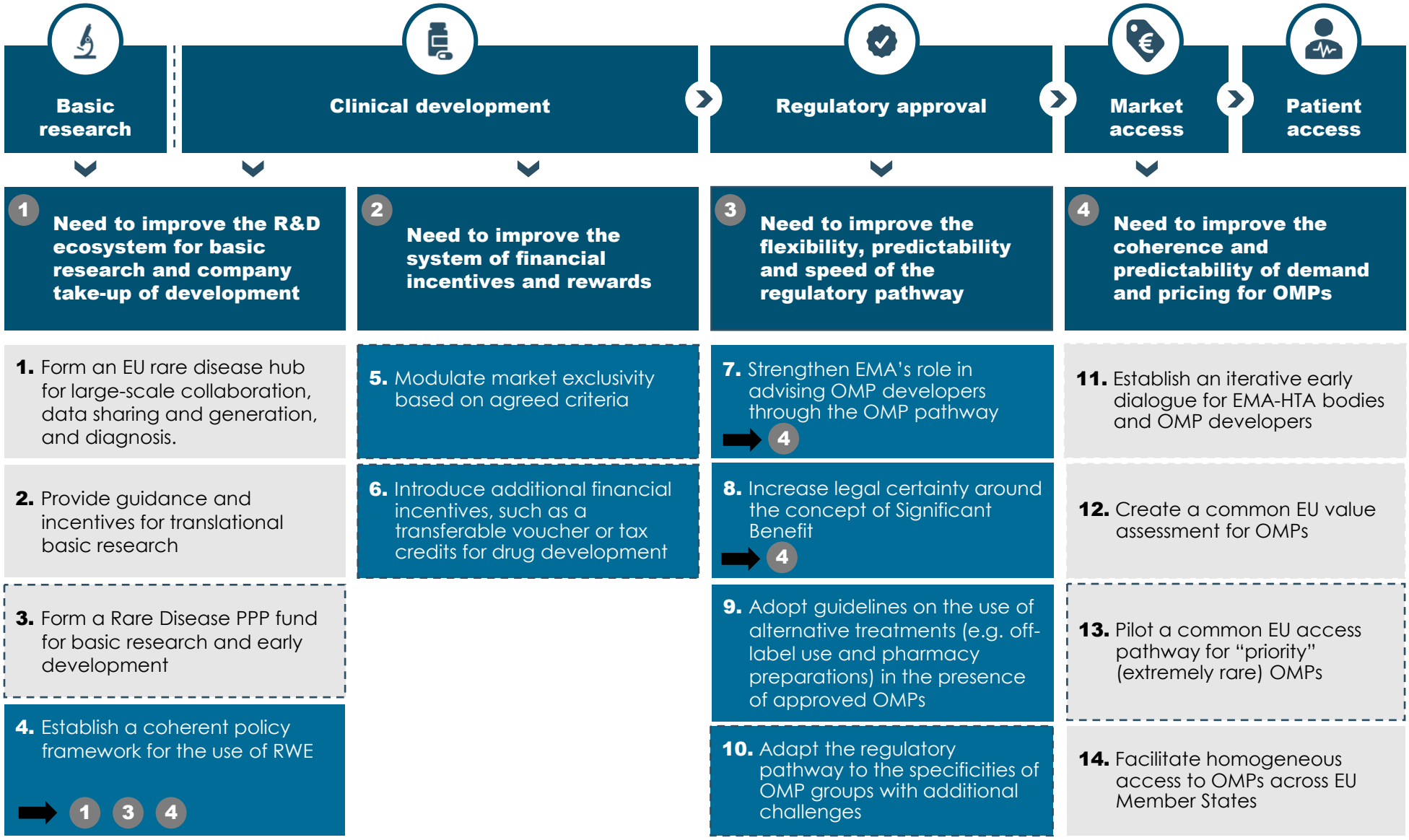
a **Conceive a holistic policy framework for the OMP development path**

b **Lead the revision from a multi-stakeholder perspective**

c **Think about policy changes from an investment perspective**

d **Ensure a competitive EU policy framework**

4 needs and 14 policy proposals



These proposals pursue or open up for a modulated approach to OMP incentives

These proposals can be addressed, partially or fully, through the revision of the OMP Regulation

And now over to our experts …

Need 1: Improving the R&D ecosystem for basic research and company take-up of development

1. Form an EU rare disease hub for large scale collaboration, sharing and generation of data and diagnosis

Bring together all actors involved in and data on rare disease onto one common platform.

EJP RD, ERNs, RD Connect, EJP Virtual platform, EU RD platform

2. Provide guidance and incentives for translation of basic research

Establish guidelines for development-ready research and appropriate incentives for basic researchers.

Orphan Drug Development Guide of the IRDiRC

Need 3: Increasing the flexibility and predictability of the OMP regulatory pathway

7. Strengthen EMA's role in advising OMP developers through the OMP pathway

- Iterative advice framework with the EMA
- strengthening role of the COMP
- improving alignment between the COMP and the CHMP

10. Adapt the regulatory pathway to the specificities of OMP groups with additional challenges

- Tailored regulatory pathway for OMPs indicated for "ultra-rare" diseases
- additional flexibility for the registration of multi-indication OMPs

Need 4: Improving the coherence and predictability of demand and pricing for OMPs

12. Create a common EU value assessment for OMPs

- A common EU framework for value assessments or (ideally) an EU-wide HTA process
- build upon and inform the early dialogue between HTAs and OMP developers
- should be designed to fit the specificities of rare diseases

- EUnetHTA

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