

Rare Disease Policy's - Industry Perspective -

Karin Blumer, Director Public Policy, Novartis
Vice-Chair joint EBE/EuropaBio Rare Disease and
Orphan Medicinal Products Task Force

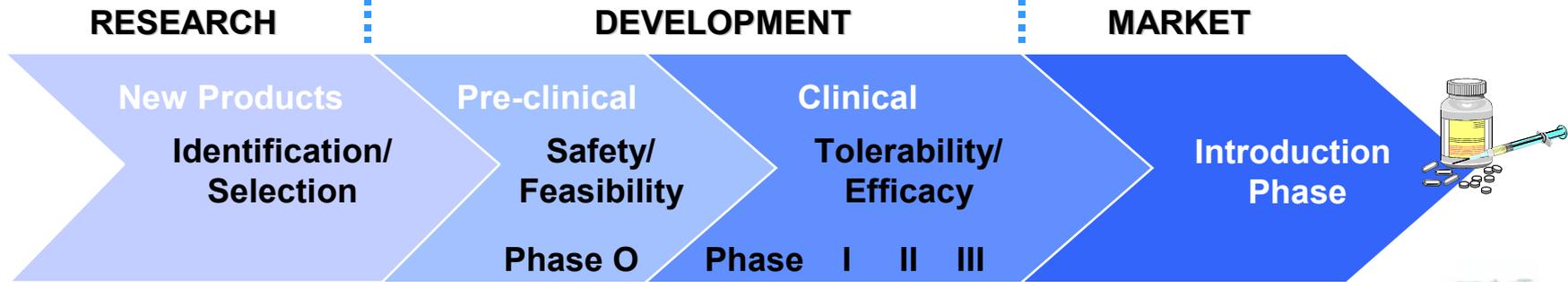
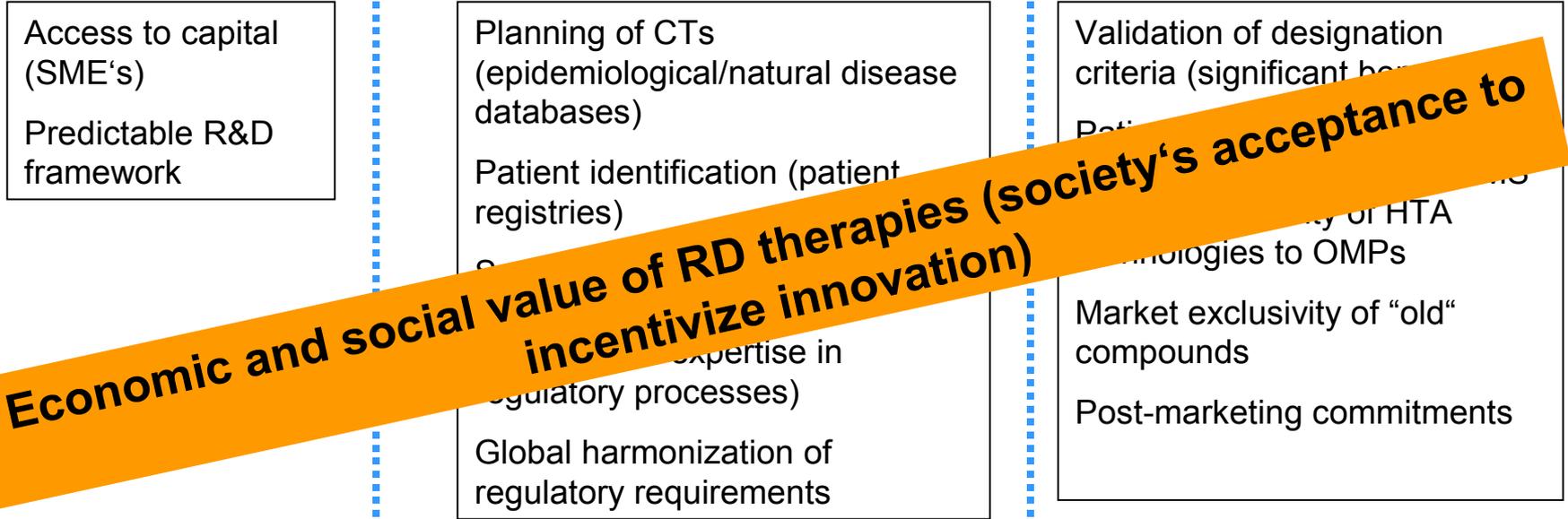


The spirit of the EU OMP Regulation

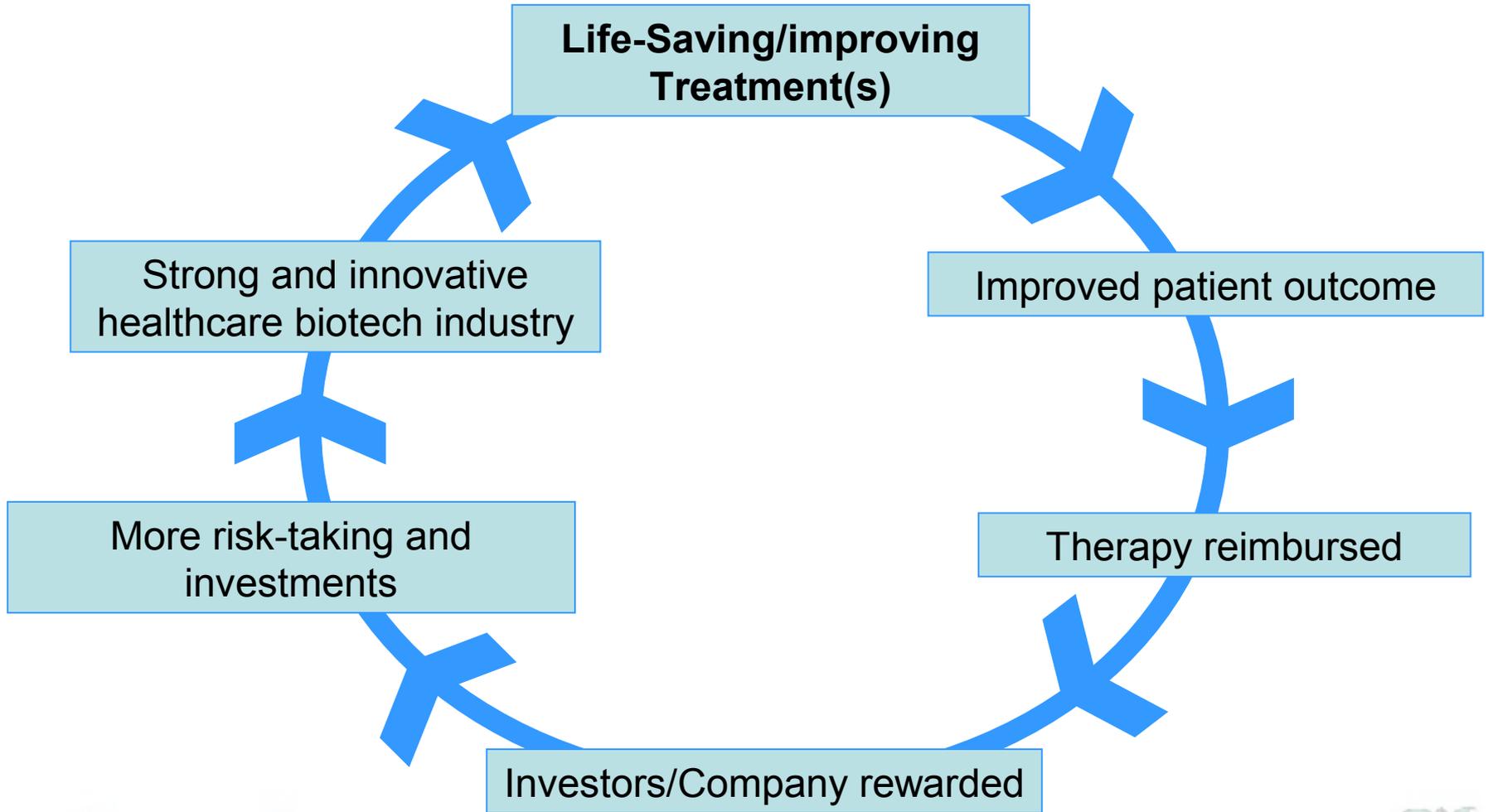
- To provide **timely and equitable access** to therapies for rare disease patients,

and
- To **balance the R&D risk** by providing economic incentives for the development of therapies
- To **bring the solidarity principle to life**: Patients with rare disorders deserve the same care and the same safety, efficacy and quality of products as patients with common diseases

Policy's impact on OMP lifecycle



Policy impact on circle of sustainability

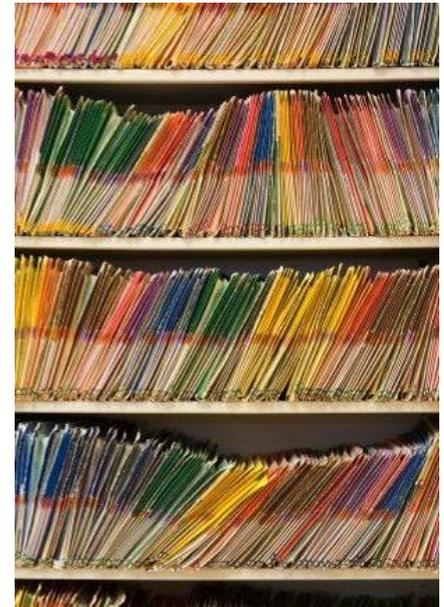


10 years after the OMP legislation...



10 years after... a success story in numbers!

- Adopted in 1999 and entered into force in April 2000
- Before 1999: 8 OMP's *avant la lettre* approved in EU
- Jan 2009: 51 OMPs approved; >500 active OMP designations
- 2001-08, average 21% per year increase in approved OMPs
- 2008: > 15,000 private or public RD-research projects* and about 2,530 clinical trials on OMPs**
- Growing number of new RD indications explored outside the OMP Regulation's framework (e.g. due to the pediatric regulation and to pathway-driven R&D efforts)



* Source Unpublished data (Office of Health Economics)

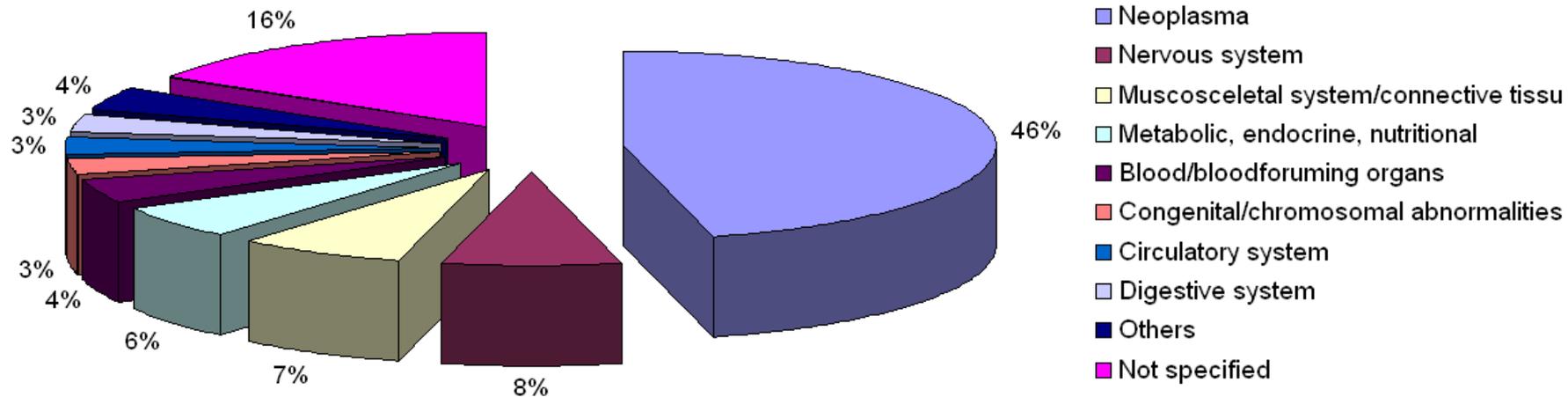
** Source: OHE/Orphanet

10 years after... New business models and strategies

Business model examples	Key activities	Average company size
<i>Development of new RD indications for “old”, off-patent compounds</i>	<i>Clinical development</i>	<i>SME, Medium and large size biotech pharma</i>
<i>Development of innovative products* for one/very few RD → no marketed products</i>	<i>R&D, pre-/clinical, development</i>	<i>Start Up SME</i>
Development and marketing of targeted innovative products for one/few RD → selected, small product portfolio	R&D, pre-/clinical, development, marketing	SME Medium size
Development/marketing of innovative RD products as part of diverse healthcare product portfolio (e.g. stipulated by Pediatric legislation, or pathway-driven R&D)	R&D, pre-/clinical development, marketing	Medium and large size biotech pharma

* “products” includes drugs, diagnostics and preventives

10 years after... More clinical trials



Source: OHE (unpublished); based on Orphanet data

But issues remain...



10 years after... Remaining issues

Common misperceptions

- OMPs address life-threatening or serious and chronic diseases for which no alternatives exist, or which have a significant benefit over existing products: **no choice of treatment**
- Rare vs. “ultra”-rare diseases: the reality is a **continuum**; research and treatment complexity increase with rarity
- There is **no avalanche of OMPs** coming, but rather a steady annual increase
- Deeper knowledge of molecular mechanisms of diseases (e.g. cancer) may lead to **re-classification and stratification of diseases**, not *per se* to “salami-slicing”, and to refinement in the use of treatments

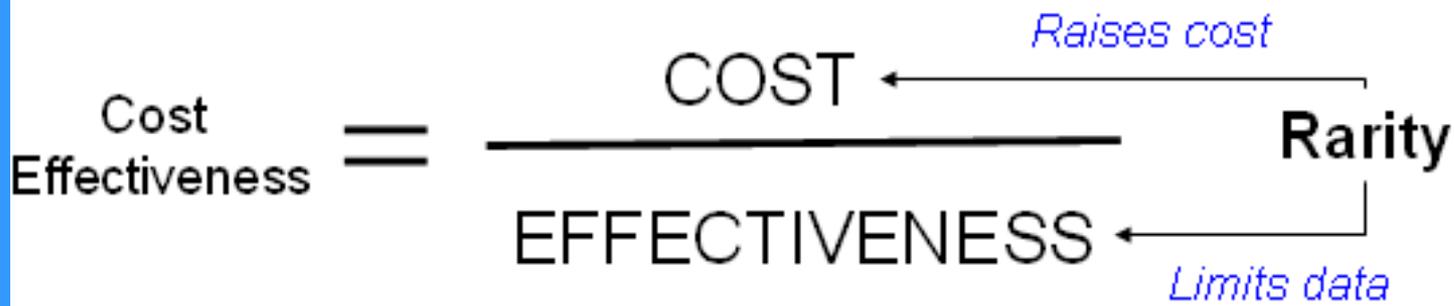
10 years after... Remaining issues

Timely and equitable access

- **Timely and equitable access** of patients to authorized OMPs remains a major issue (Commission Communication on RD; 11/08)
- **Long** discussions on price and reimbursement in some countries
- **Country size** seems to impact access (smaller countries → more difficult the access; Eurordis); thus **regionalization** of healthcare makes access even more difficult
- **HTA applicability** for (ultra-)orphan drugs? For OMPs where it is possible: can assessment happen at EU level, appraise locally? Patients and their representatives should participate in the assessments to ensure that HTA procedures are transparent, timely and include the wider social costs and benefits
- **Added clinical value assessment of OMPs** and **increased collaboration between MS and EU authorities** (proposed by EC Communication) is a promising way forward

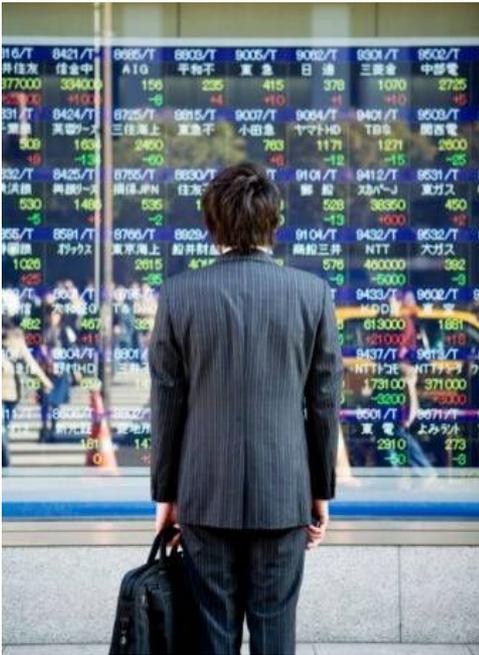
10 years after... Remaining issues

Rarity and Cost-effectiveness



10 years after... Remaining issues

Market's role in continuous and sustainable development of OMPs



- Large companies have greater ability to absorb risk than small companies
- Many SMEs are highly vulnerable to economic distortions (may have only 1 drug in development for one indication)
- However, **NO** company or investor will support the development of a drug if they know there is no market

Business Model Must Be Sustainable

10 years after... Fostering the development of National Action Plans as a way to address many issues

National Action Plans (NAPs) need to aim at:

- Improving health outcomes and quality of life for EU rare disease patients regardless of where they live; and
- Facilitating R&D and delivery of innovative, life-saving treatments
 - By benefiting both those patients where a treatment exists and those patients whose condition does not (yet) have a treatment
 - By initially focusing on a limited number of key activities to deliver quick and effective results, with the intention to build on these over the coming years
 - By ultimately supporting the creation of multi-national patient registries, natural disease databases and centers of excellence
 - By creating multi-stakeholder advisory groups involving national authorities, clinicians, researchers, patients, payers and industry, to help to the set-up and implementation of national action plans



10 years after... An EU political momentum to be happy about

- The **EU Commission Communication on Rare Diseases: Europe's Challenges**, published in November 2008
- The culmination of a consultation process where the Joint EBE/EuropaBio Task Force inputted
- The **EU Council Recommendations on action in the field of rare diseases**, published on 9 June
- Positively welcomed by the Joint Task Force!



What about the next 10 years?

Access to capital
(SME's)

Predictable R&D
framework

Planning of CTs
(epidemiological/natural disease
databases)

Patient identification (patient
registries)

Specialized expertise
of excellence;
independent expertise in
regulatory processes)

Global harmonization of
regulatory requirements

Validation of designation
criteria (significant benefit)

Patient centered value debates at MS
level; applicability of HTA
technologies to OMPs

New indications for old
compounds

Post-marketing commitments

Many open issues await targeted, effective policy action

RESEARCH

New Products

Identification/
Selection

DEVELOPMENT

Pre-clinical

Safety/
Feasibility

Phase O

Clinical

Tolerability/
Efficacy

Phase I II III

MARKET

Introduction
Phase



In the next 10 years... Continuing the efforts



Industry is living up to the expectations of patients, society and legislators.

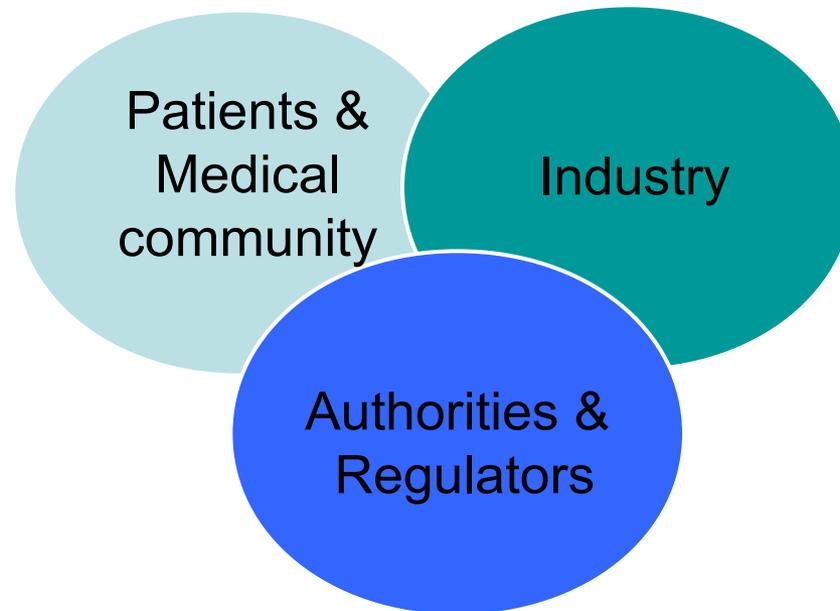
We stay committed to deliver innovative, safe and effective therapies to rare disease patients across the EU

But

- Rare diseases are by nature “global diseases“
- Multi-national action as provided by the OD Regulation is essential
- To secure the momentum and drive future developments there needs to:
 - Ensure complementarity with national level initiatives (80% of all EU clinical trials on OMPs are conducted in countries with a national plan)
 - Increase global policy alignment
- National action plans are a key element

Industry remains a part of the solution in a system of shared responsibilities

- Create awareness for medical need and patient rights
- Provide and request information
- Establish networks and communities
- R&D for new, safe&effective drugs
- Create competitive environment
- Compassionate use programs
- Share knowledge



- Provide incentives → foster innovation
- Create framework for R&D
- Grant timely and equal access to treatment
- Adapt regulations to high and different needs
- Ensure sustainability of healthcare systems