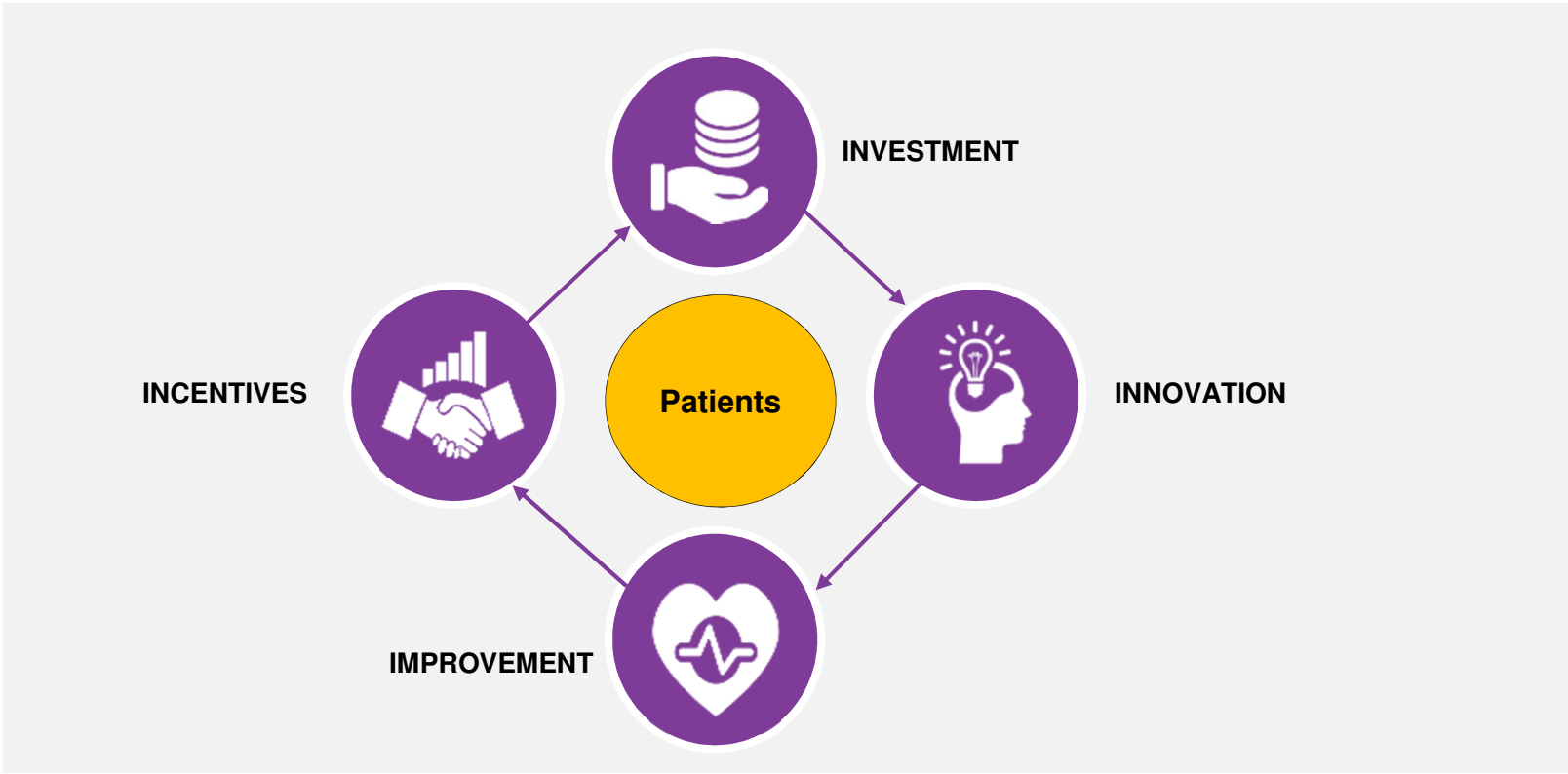

INVESTMENT
INNOVATION
IMPROVEMENT
INCENTIVES
Patients

The Impact of the Orphan Medicines Regulation

Dynamics of therapy development for Rare Diseases

CRA Charles River Associates

11th May 2018

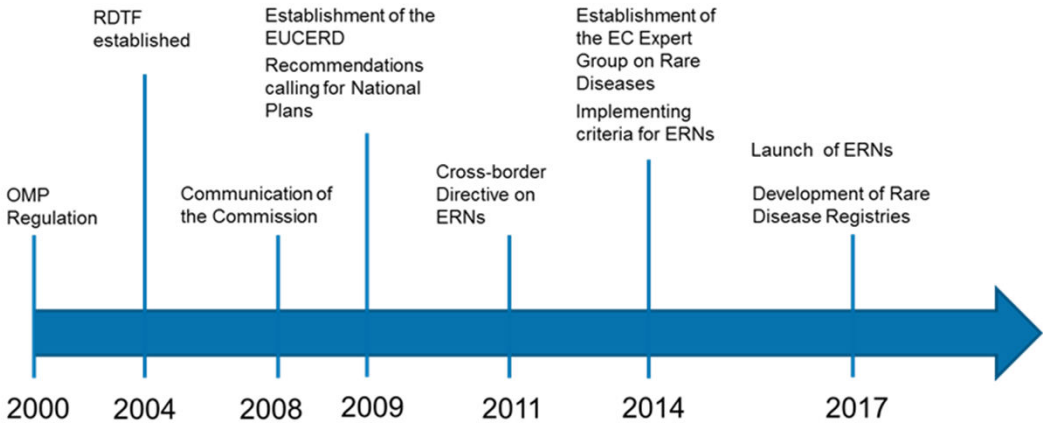
Agenda

- Recent research on the value of OMP regulation
- Key findings
- Conclusions

Recent research on the role of OMP regulation

- There have been a number of studies examining the impact of OMP rules over the last 16 years.
- CRA has researched the broader societal and economic impact of the orphan medicines and rare disease policies drawing on literature and interviews with a range of stakeholders (patient groups, professional bodies and industry)

Timeline of EU policies for Rare diseases

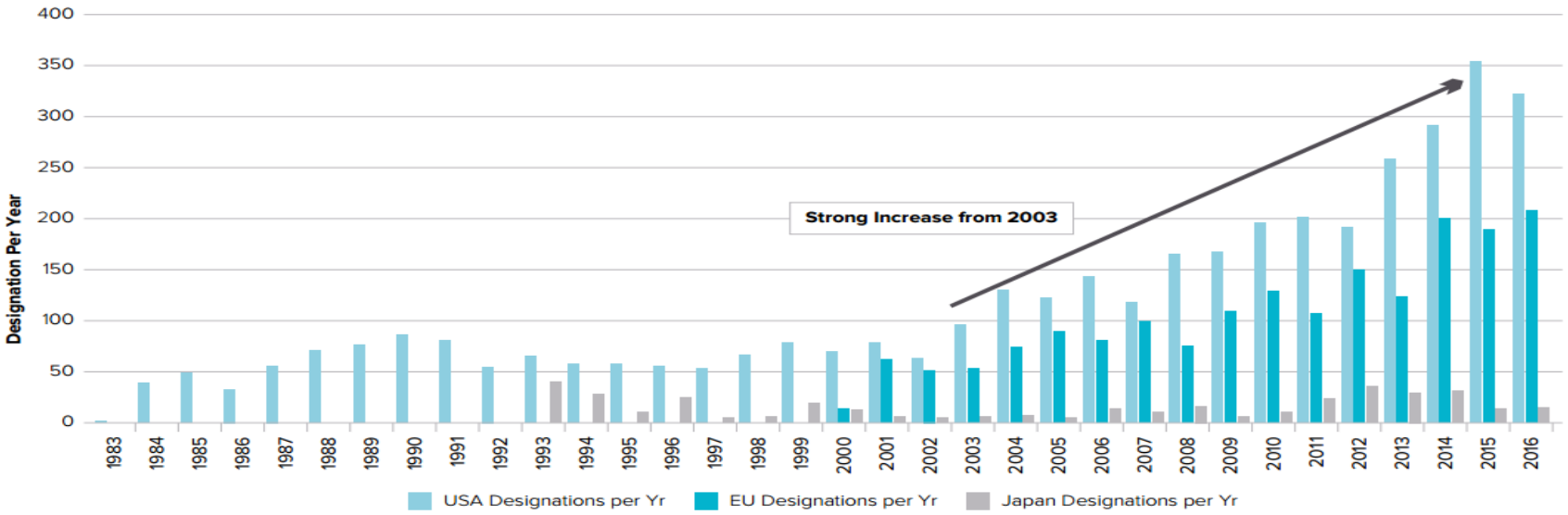


Investment - European R&D environment

- There is a favourable environment for research and development and the **establishment of research networks**
- The number of **scientific publications** on RD has grown significantly over the years
- There is a positive trend in the **basic research projects** being undertaken in the EU
- **Companies** specialising in rare diseases or with focused rare disease BUs have increased considerably compared to the past
- Within the first decade following the implementation of the OMP Regulation, there was a substantial increase in **new SME biotech** companies across Europe

Direct indicators to measure impact of the OMP Regulation

- To date, the EC had granted **1,952 orphan designations**, and **142 OMPs** had obtained marketing authorisation
- There was also a significant increase in the growth of global orphan medicines designations shortly after 2000
- There has been a **significant increase** in European on-going clinical trials for RD

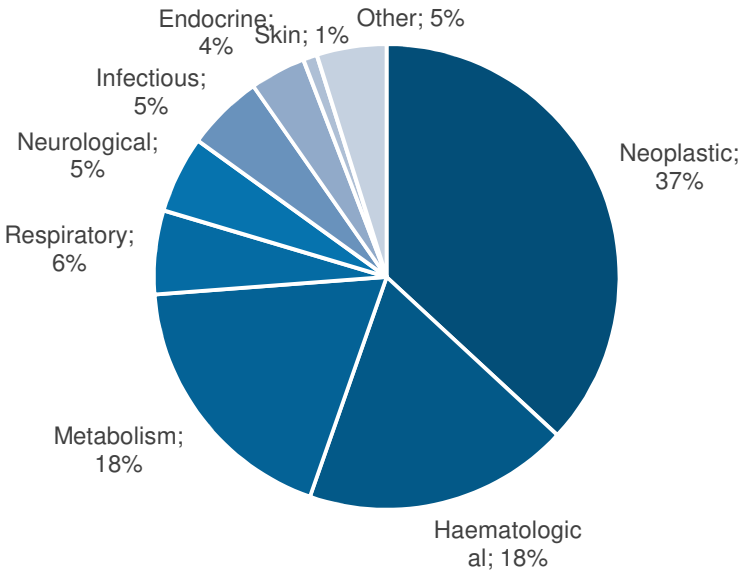


Source: EvaluatePharma

Direct indicators to measure impact of the OMP Regulation

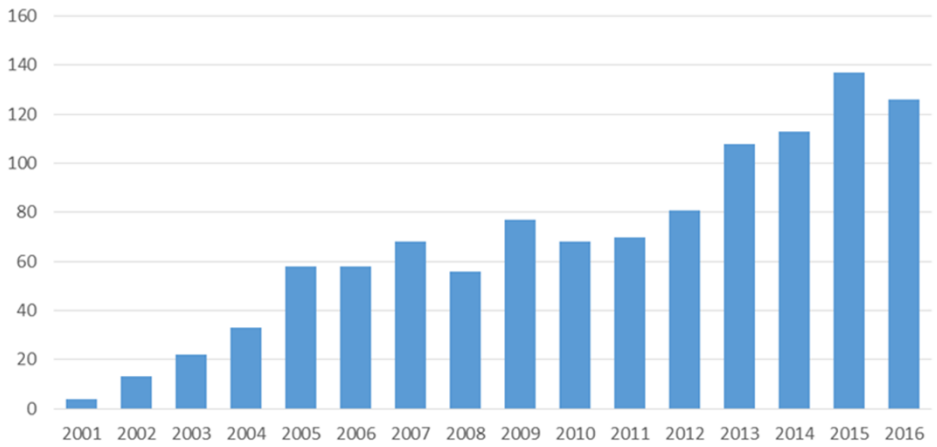
- OMPs target a **wide range** of disease areas
- Recent studies show that an increasing number of designations are for conditions affecting **fewer than 1 in 10,000 patients**

Analysis of OMP approvals (2000–2015)



Source: Giannuzzi et al.

Protocol-assistance and follow-up requests received by the EMA

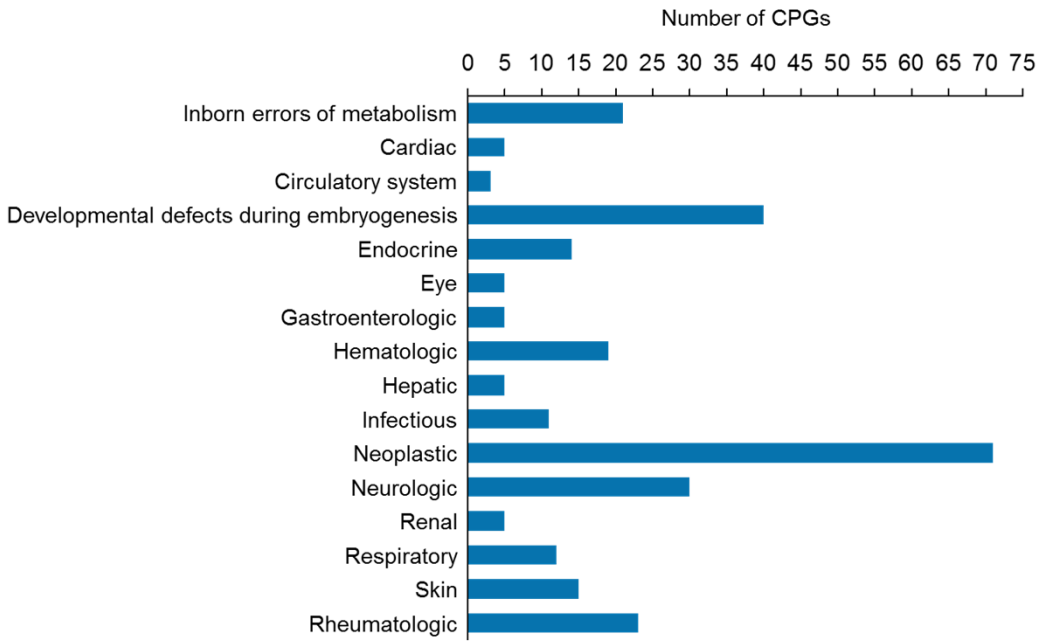


Source: European Medicines Agency

Improvements – Treatment provided by healthcare professionals

- Increased research, evidence and medicines has **increased the awareness** of rare diseases significantly
- **New resources** are now available for physicians
  - Diagnostic tests,
  - Patient registries
  - Clinical practice guidelines for RD
- **New infrastructures**
  - European Reference Networks

Clinical practice guidelines distribution on Orphanet by medical speciality



Source: Pavan et al.

Improvements – Health and quality of life

- Patient organisations report that medicines developed using the incentives foreseen in the OMP regulation have **significantly improved patients' health and quality of life**, and decreased their reliance on supportive care
  - Impact on life expectancy :Some treatments give patients the opportunity to enjoy a normal life expectancy, or provide a significant improvement in survival.
  - Impact on quality of life: Some treatments reduce health-related issues that limit normal activities, thus improving patients' quality of life.

  CAVEAT The heterogeneity of the rare diseases therapeutic area mean that it is impossible to present aggregate improvements and the evidence is derived from specific disease examples in the following categories
    - **rare cancers**
    - **life-long genetic conditions**

However the **access** to care and treatment remains a concern in many European countries

Conclusions

- Developing quantitative evidence on the impact of the OMP and rare disease regulations is challenging
  - There might be other factors than the set of regulations and measures influencing the same metrics
  - We are pooling data from very different sources

- However, we find compelling indications of a **direct, societal and economic impact** of the OMP regulation and rare disease policies
- Significant unmet needs remains and hence there is an even greater opportunity to deliver significant benefits in the future

REFERENCE
https://www.efpia.eu/media/361828/an-evaluation-of-the-economic-and-societal-impact.pdf