A continuum approach to evidence generation in post-approval phase linked to healthcare budget spending

D. Ardigò, MD PhD

R&D Rare Disease Unit Head
Corporate Research & Development
Chiesi Farmaceutici S.p.A.

Chairman Therapies Scientific Committee
International Rare Disease Research Consortium (IRDiRC)

Disclaimer

The opinions expressed in this presentation are the results of my own personal perspective and should NOT be considered as representative of the opinions and views of Chiesi Farmaceutici SpA or IRDiRC.

Is this sustainable?

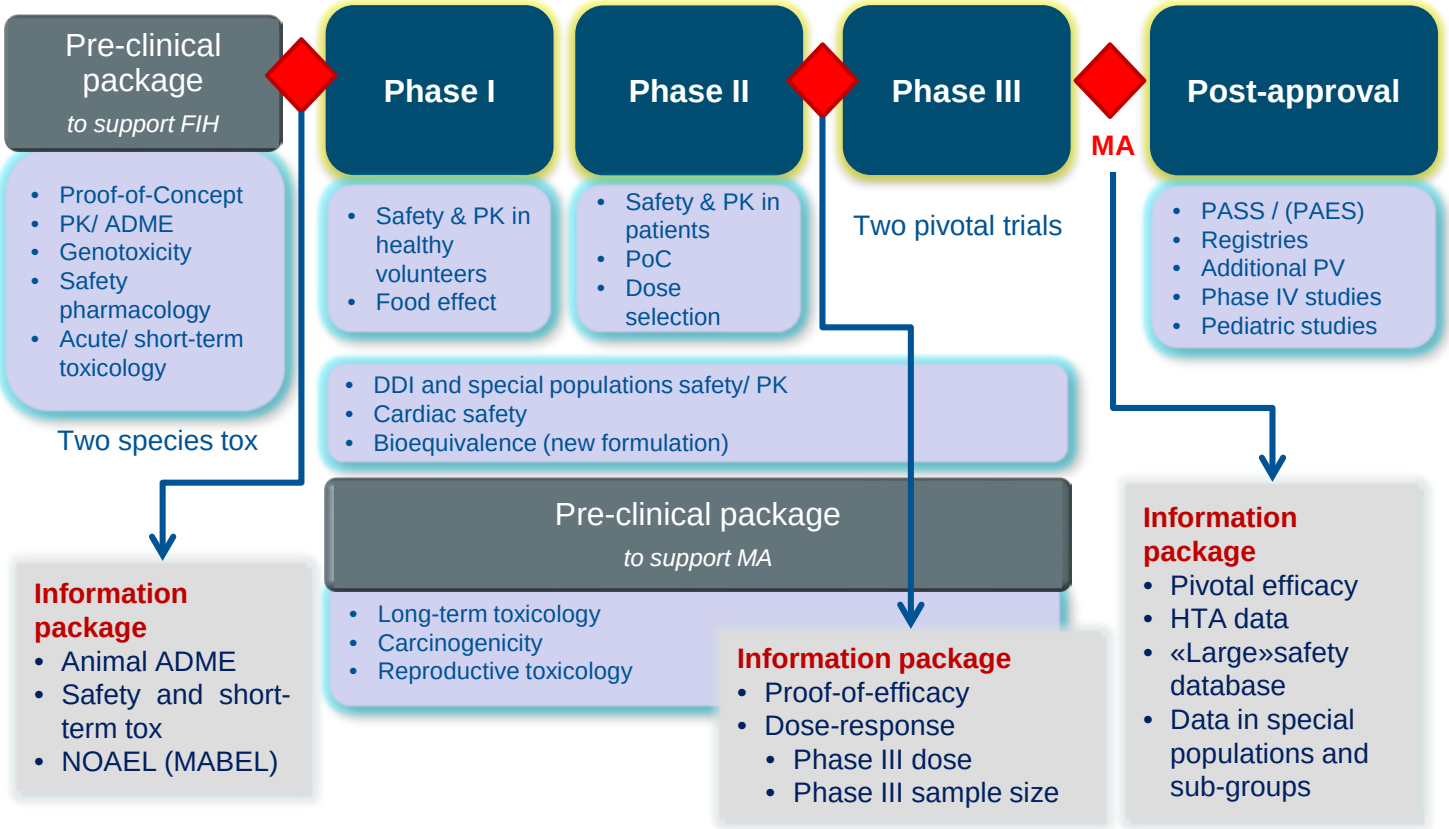
IRDiRC
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

GOAL 2:
More than 1000 new drugs in the next 10 years to treat rare diseases

TECHNICAL SUSTAINABILITY

ECONOMIC SUSTAINABILITY

Development of a drug (small molecule, large indications)



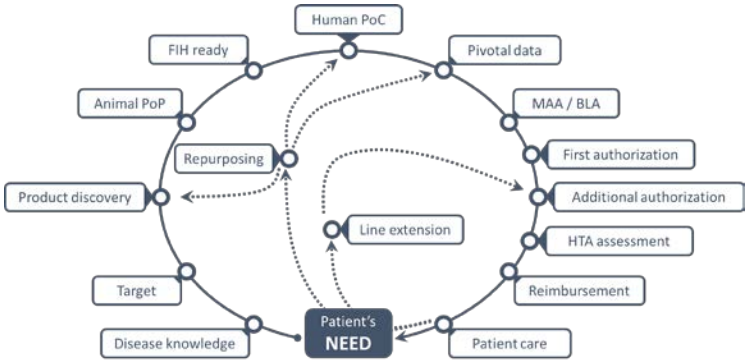
- Consolidated
- Based on risk management and gate reviews
- Almost linear progressive increase in costs
- Long-term predictability

Consequences of rarity in drug development

- Less understanding of disease mechanisms and progression
- Less confidence in PoC and dose
- Lower statistical power in pivotal studies
- Lower confidence in traditional end-points
- “One shot only”

- Risk Management
- Post-approval data
- Value uncertainty

Where to act…



- A better use of the tools that we already have
  - EJP-RD
  - IRDiRC Drug Development guide
  - Repurposing
- Integrate care, research, and development
  - Disease registries
  - Clinical and research networks
- New models to fund access to innovative treatments
  - Reimbursement models and payment schemes
  - Centralized HTA

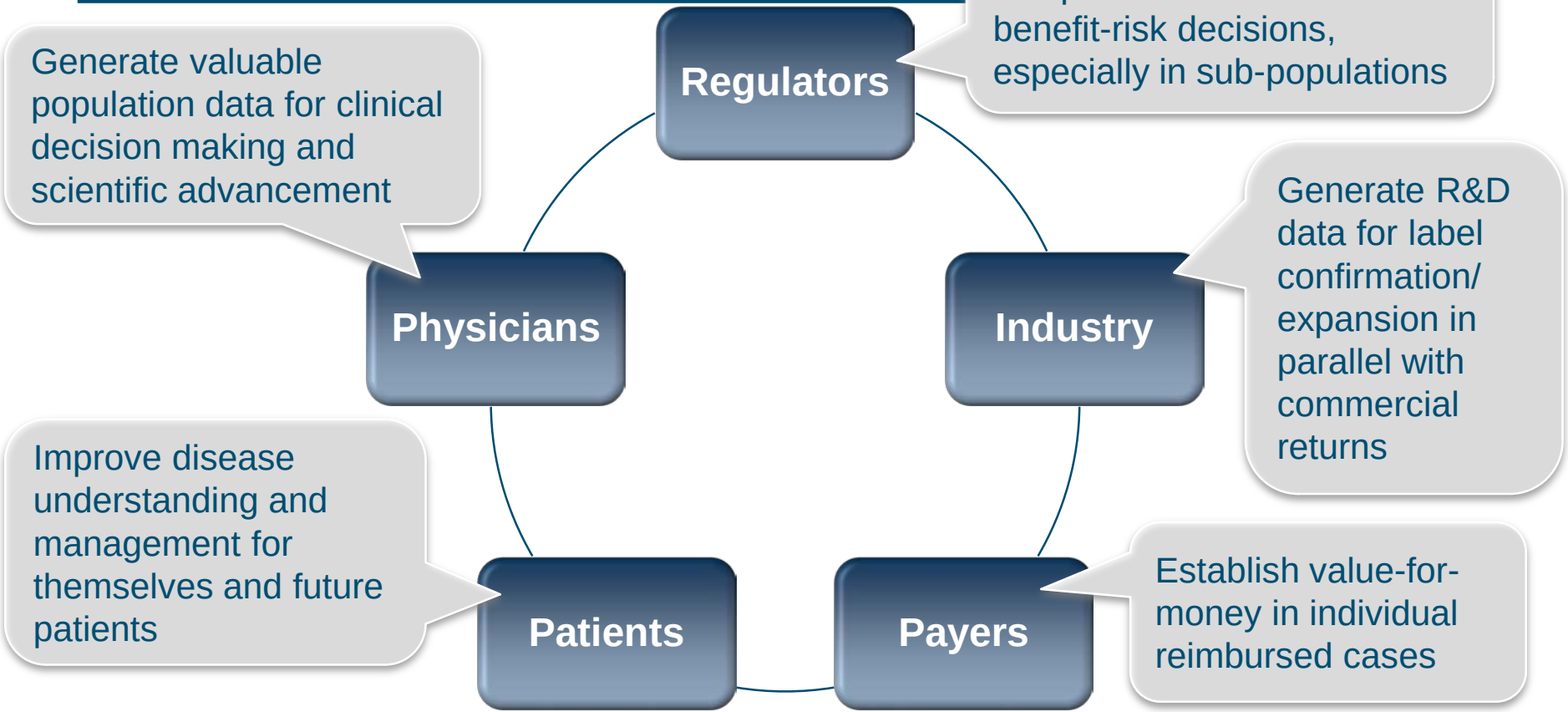
Registries and data creation

A registry is...

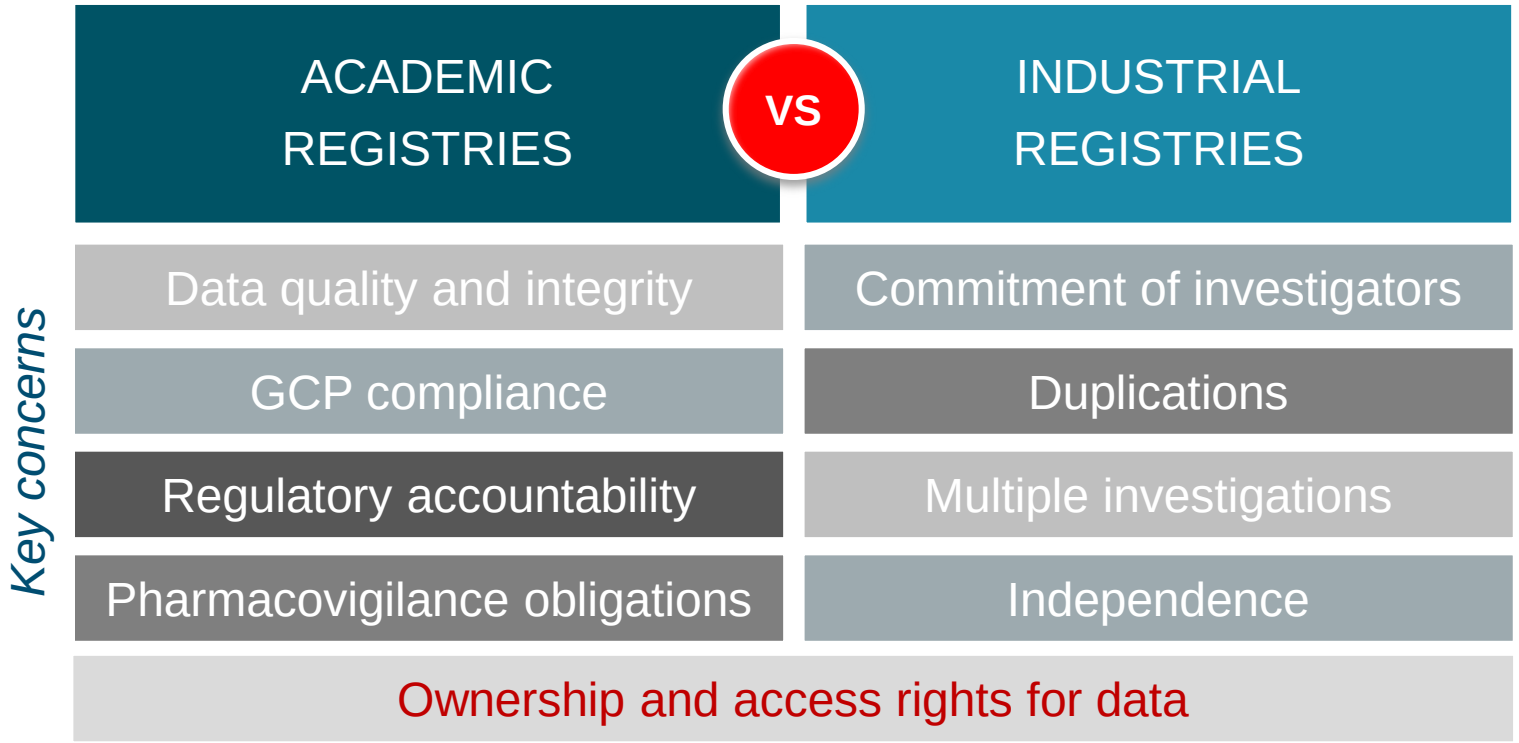
"A patient registry is an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more predetermined scientific, clinical, or policy purposes."

Gliklich R, Dreyer N, editors. Registries for evaluating patient outcomes: a user's guide (prepared by Outcome DEcIDE Center [Outcome Sciences, Inc. d/b/a Outcome] under Contract No. HHSA29020050035I TO2). AHRQ Publication No. 10-EHC049. 2nd ed. Rockville: Agency for Healthcare Research and Quality; 2010.

Expectations…



Types of registries



Post-approval Registry / PASS / PAES

- **It is NOT a traditional study**
  - Living organism
  - Multiple stakeholders
- **It IS a full clinical trial**
  - Detailed protocol
  - Real study (CRF, monitoring, etc.)
- **It is an heavy study**
  - Interim analysis (every year)
  - Reconciliation between database and pharmaco-vigilance system
  - Costly study (number of countries and centers “disproportionate” to the number of patients)

A vision for the future: a multi-sided reality…