

Agenda of the session

- Overview of the major bottlenecks in translating research
Diego Ardigo, Chiesi Group
- How to get research done on your rare disease conditions
Daniel Lewi, CATS Foundation
- How to develop and adapt a co-design model to design research
Alison Metcalfe, Kings College London
- How to make exploitable research
Lucia Monaco, Fondazione Telethon
- Crack it challenges from the industry perspective
Jon Timmis, SimOnics

PANEL DISCUSSION



Overview of the major bottlenecks in translating research

ERDC 2018 – Session 102

Diego Ardigó, MD PhD

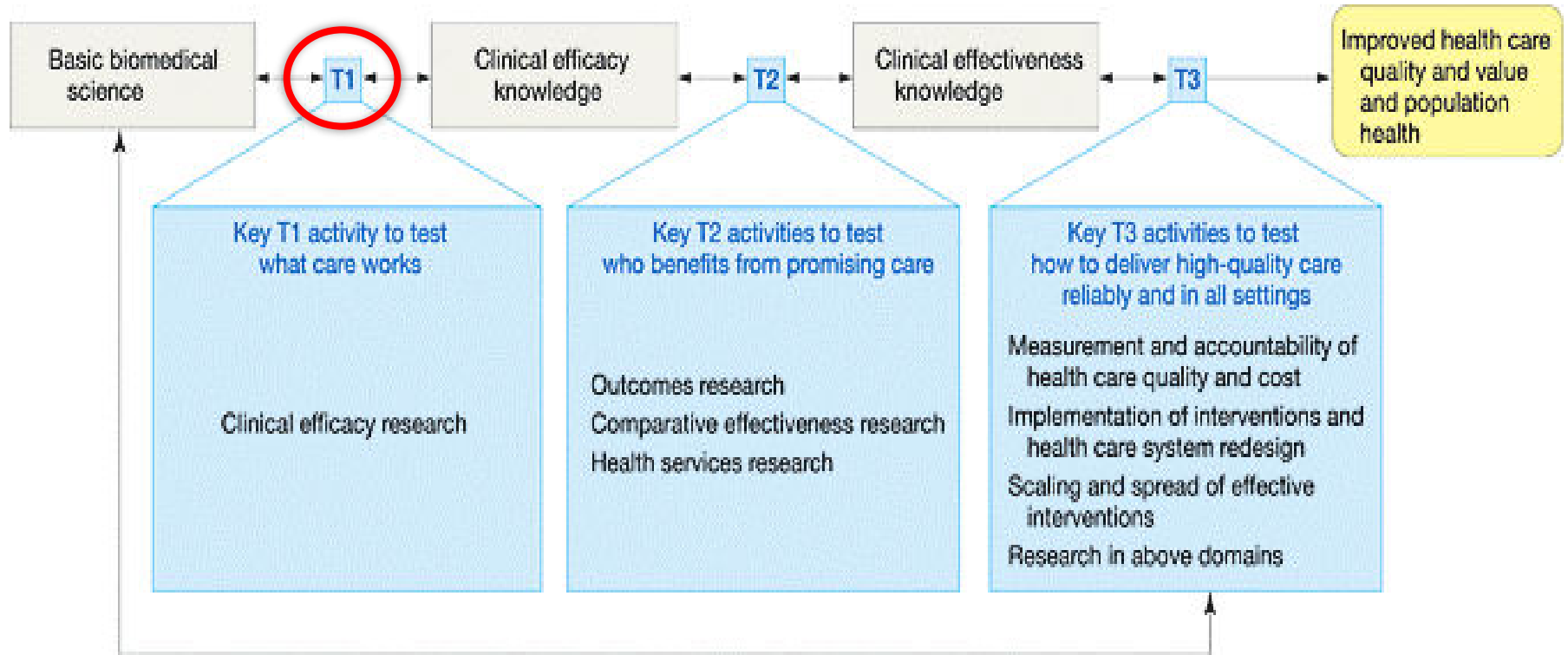
*R&D Rare Diseases Unit Head
Chiesi Group (Chiesi Farmaceutici S.p.A.)*

*Therapies Scientific Committee Chairman
International Rare Disease Research Consortium*

Translational research

The “translation” of basic scientific findings (in a laboratory setting) into potential treatments for diseases

The translational research concept and focus



Dougherty, D. et al. JAMA 2008;299:2319-2321

Efficiency of translation

Academic translation

Average success rate to “translate” laboratory findings into fruitful clinical trials is ~8%

Mak S et al. Am J Transl Res. 2014

Industrial development

Only 1 over 5-10 drugs entering into clinical development is deemed to enter into the market

DiMasi JA et al. J Health Econ 2003.

Why translation fails

- Animal models do not adequately represent complex human physiology
 - Predictive Efficacy
 - Predictive Toxicology
- Understanding, robustness, and quality of the model (or the assay)
- Sharing of information (including publication bias)
- Low reproducibility of laboratory findings
- Failure of clinical trials to prove efficacies
- (Specific complexity of rare diseases)

Why translation is so difficult

- Low hanging fruits are taken
- Increasing requirements
 - Regulatory (efficacy and safety)
 - Payers (value)
 - Medical practice
- Cost, time and need for committed resources
- Increased complexity and portfolio of skills
- “Battle” of cultures and interests

“The disincentives in academic medicine for top laboratory scientists and clinical investigators to commit to the long-term team research necessary for effective translational research are formidable.”

Richard Simon, National Cancer Institute (Bethesda, MD, US), Exp Rev Mol Med 2010

Key success factors



Knowledge of disease and predictive animal models

Strategic mind (start from the end)

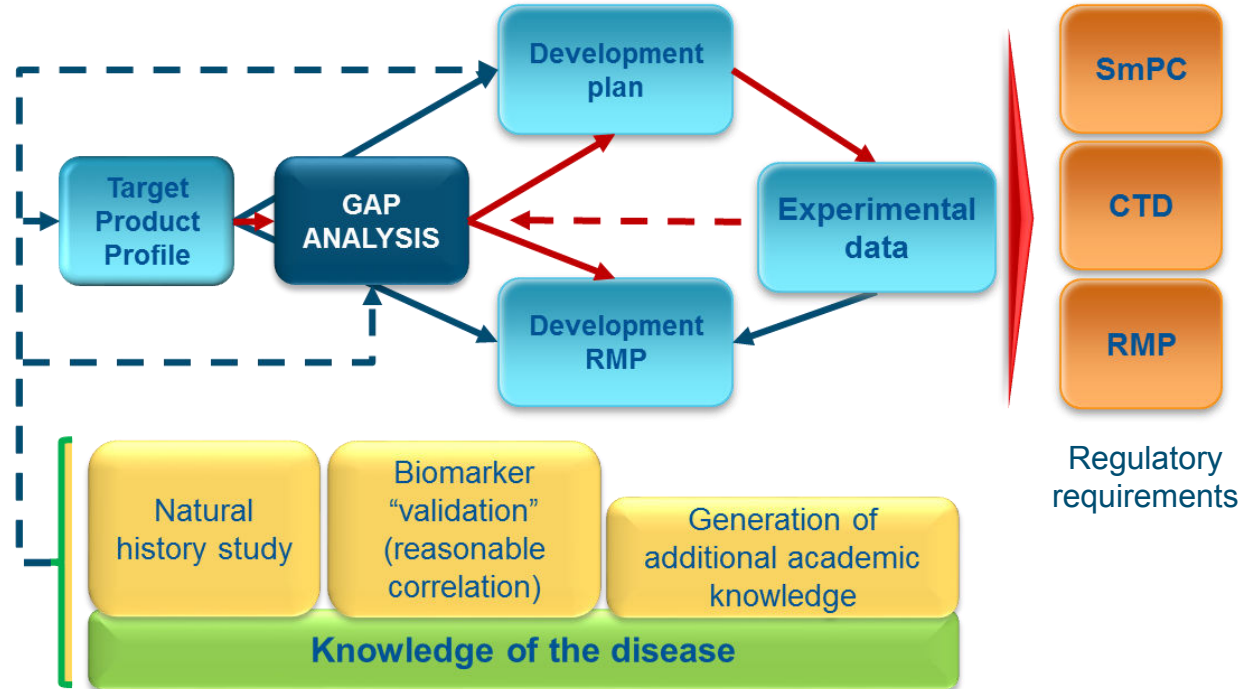
Strategic approach to translation into drug development

Target Product Profile

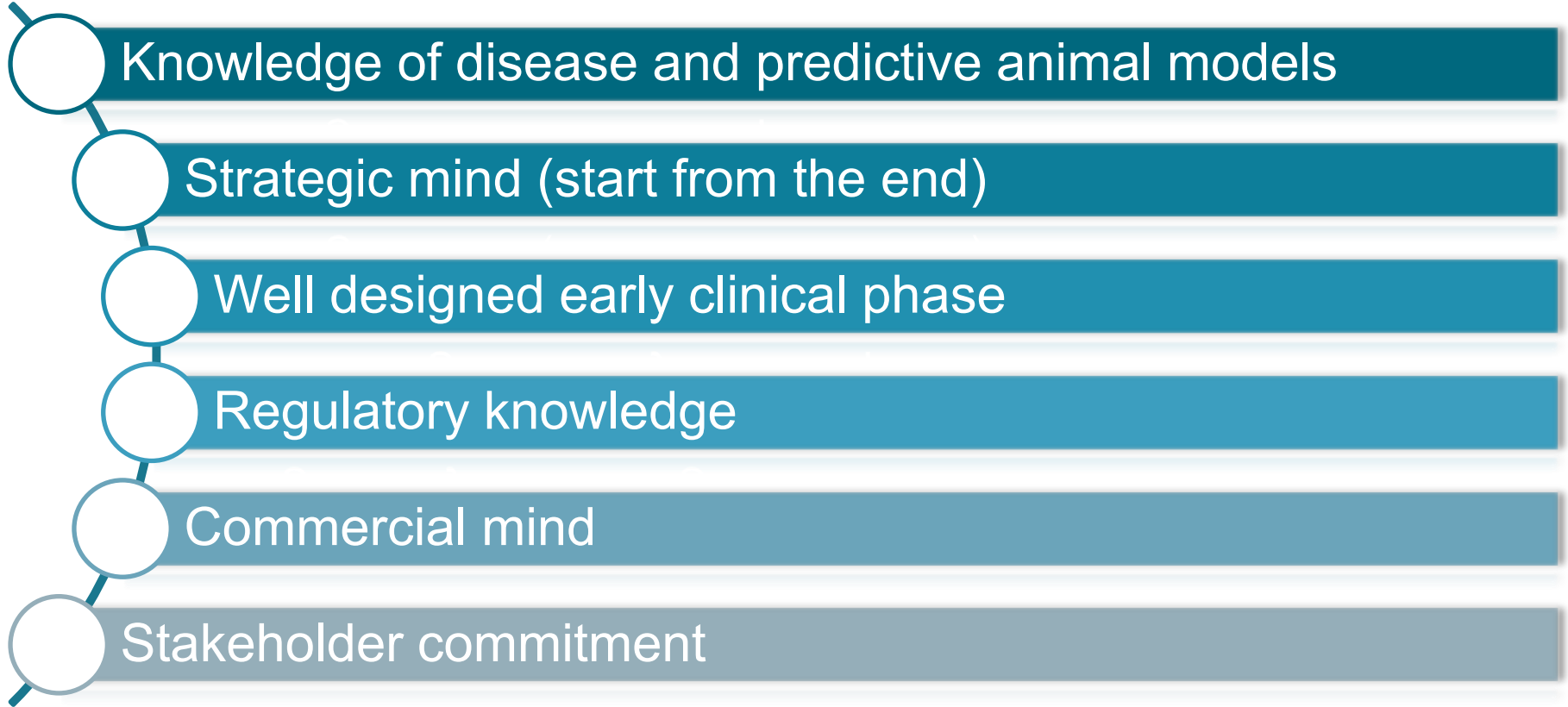
1. Indication/ Patient Population
2. Value proposition/ Place in therapy
3. Dose, route & schedule of administration
4. Efficacy (label claims)
5. Safety and tolerability
6. Partnering strategy (how and when during development)
7. IP and branding strategy
8. Geographic scope and Estimated filing/launch dates
9. No go criteria

Knowledge Gaps

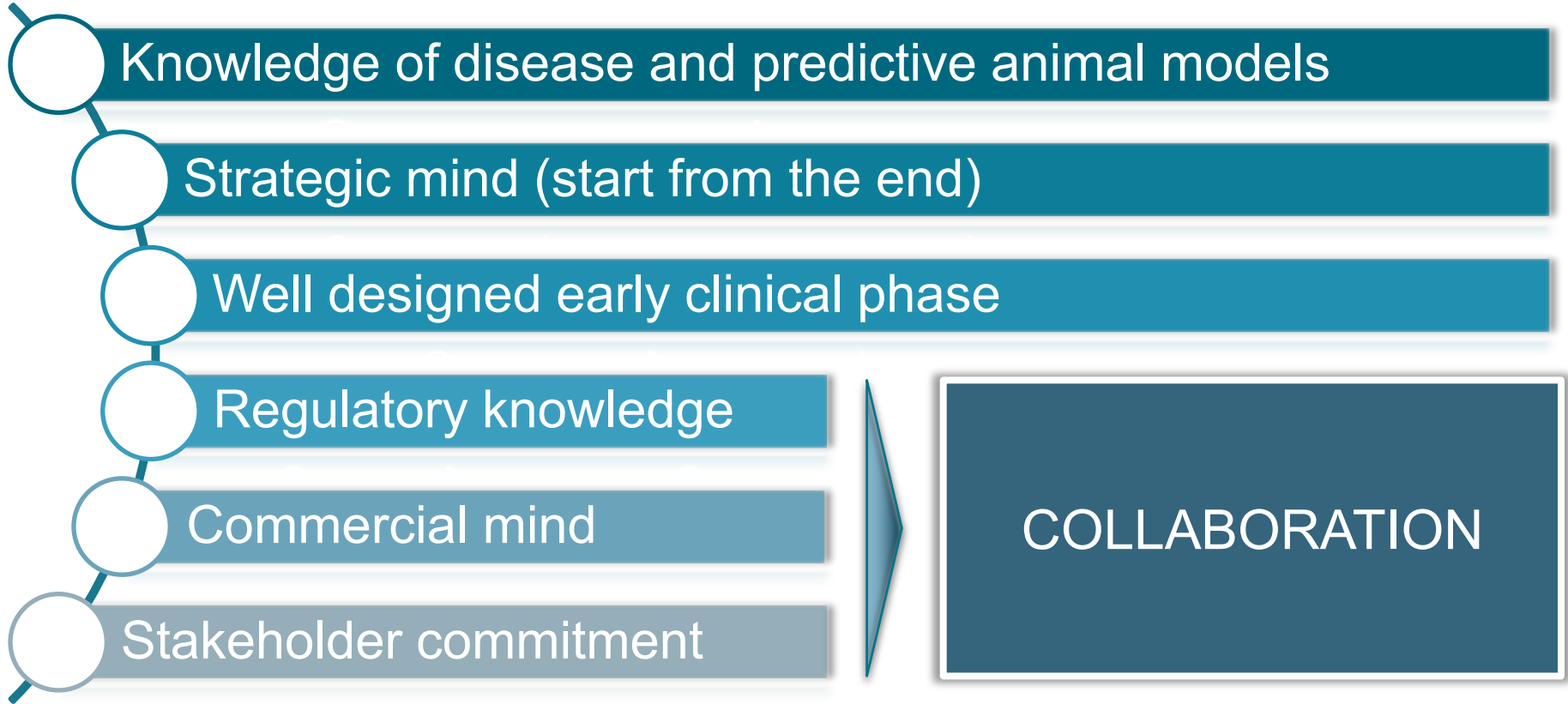
Risks and their management



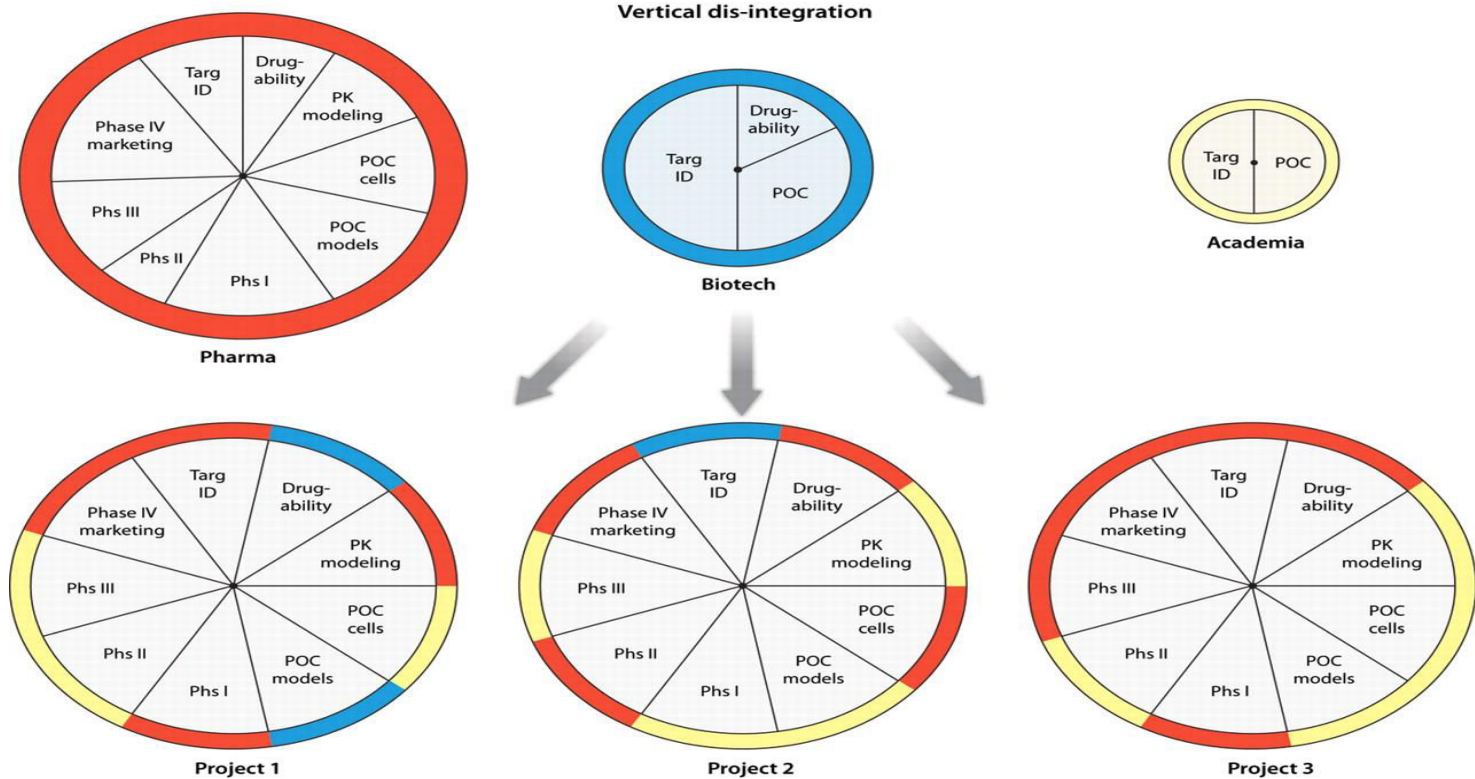
Key success factors



How to overcome the translation valley of death?



Future Vision: Integration/Collaboration



Sci Transl Med 7 April 2010: Vol. 2, Issue 26, p. 26cm12