



Drug repurposing

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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*

Credits

Most of today's concepts come from discussions held in the following occasions

- International Rare Diseases Research Consortium (IRDiRC)
 - Data Mining and Repurposing Task Force
- Eurordis Roundtable of Companies 2018
 - Breakout session on Drug Repurposing

Mostly with the following people

- Virginie Hivert (Eurordis)
- Daniel O'Connor (MHRA, UK)

(although everything expressed in this presentation reflects my personal opinion)

Is this sustainable?

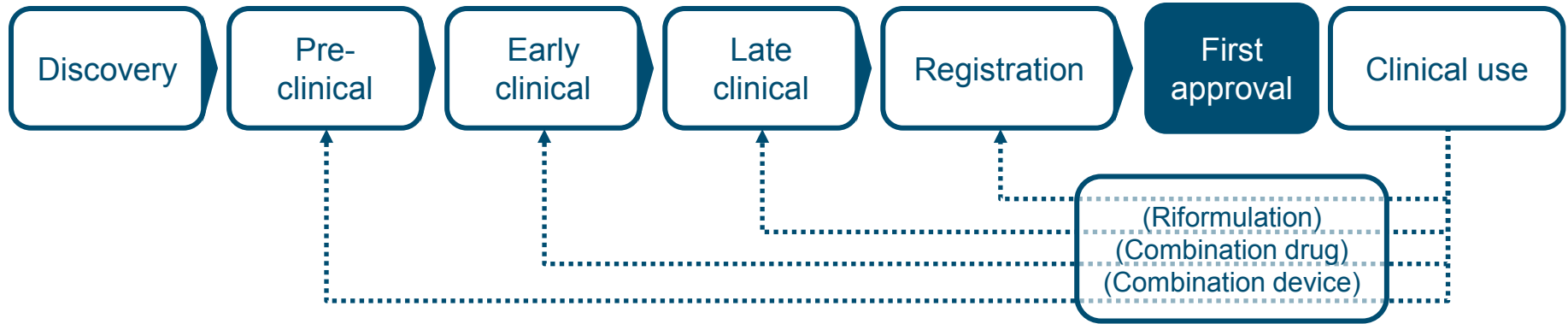
**More than 1000
new drugs in the
next 10 years to
treat rare diseases**



**TECHNICAL
SUSTAINABILITY**

**ECONOMIC
SUSTAINABILITY**

What is repurposing



New use for an existing drug in an indication outside of the scope of the initial indication

Main focus for today

- Approved drug with expired protections (IP, regulatory, etc.)
- With generic products available
- To be used in a different indication (possibly a different disease)
- New protection cannot be generated in a traditional way (e.g. change in formulation)
- Needing additional data to support the indication

Repurposers: who can do it...

- Drug manufacturers
- Academic researchers
- Non-profit organizations
- Patient organizations
- Institutional organizations (health institutes, research funders, etc.)

To repurpose or not to repurpose ...

WHY YES

Based on medical need

Faster development

Cheaper development

Lower development risk

Clinical acceptance



WHAT OBSTACLES

Economic return

Funding of research

Uncertainties around the data requirement

Uncertainties around the value of the indication

Knowledge on drug development

Key success factors



Better collection of real-world clinical data

Systematic collection of clinical data on off-label use

Better integration of existing research data

A sustainable business model

How can we incentivize repurposing?

- ~~Market protection~~
- ~~Higher price~~
- Tax credit [limitations due to national vs EU level]
- Vouchers [along the line of Priority Review Voucher in US]
- Grants/ dedicated funds for research [to cover costs]
- Prescription restrictions and control [very difficult to enforce]
- Partnership: sharing of costs – risks – returns