Some Industry Perspectives

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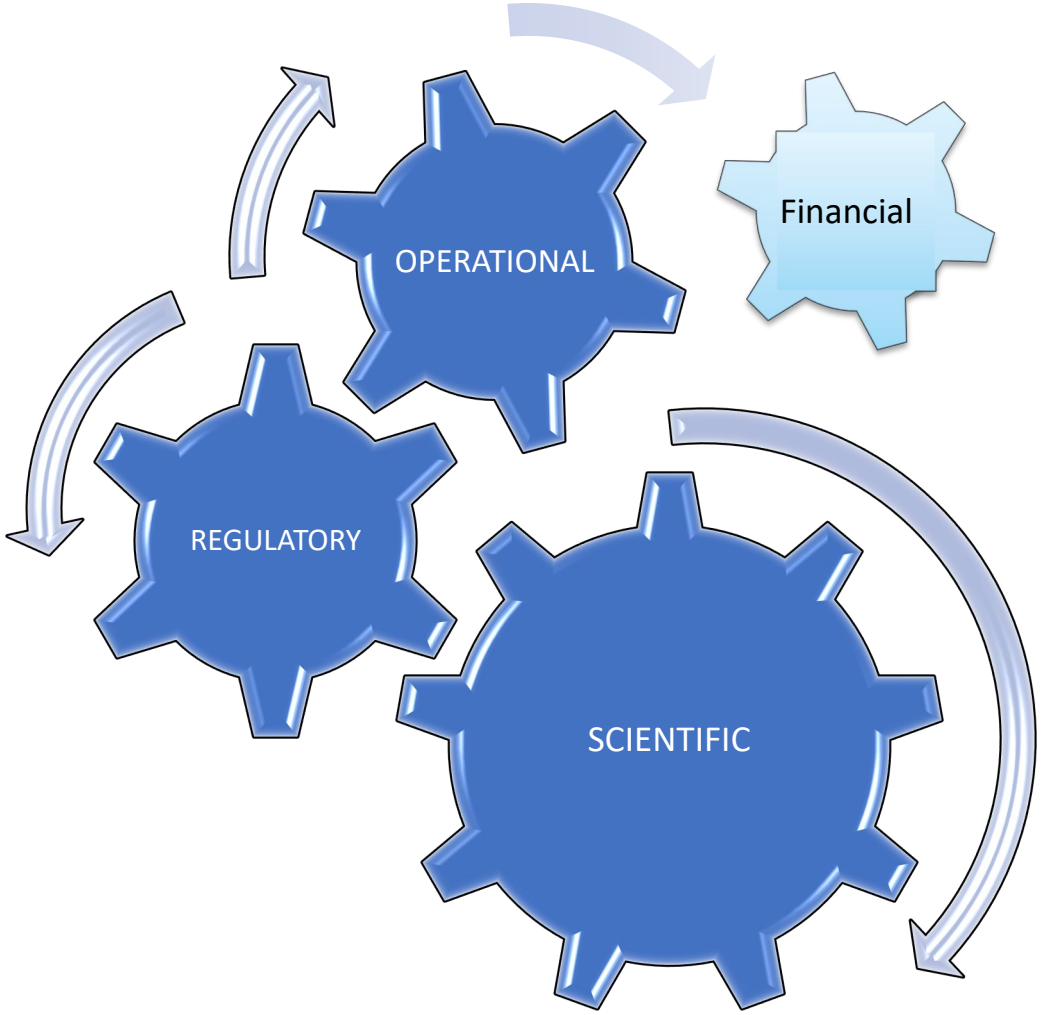
Disclosure

These are my views and opinions and they do not represent the views and opinions of Johnson and Johnson or Janssen Pharmaceuticals

What is needed?

- Scientific
  - N+3 studies
  - PK/PD extrapolation
- Regulatory
  - Registries/natural history studies
  - Contemporaneous controls
- Operational
  - 1 arm trials
  - Established infrastructure
- Financial
  - Efficiency
  - Incentives

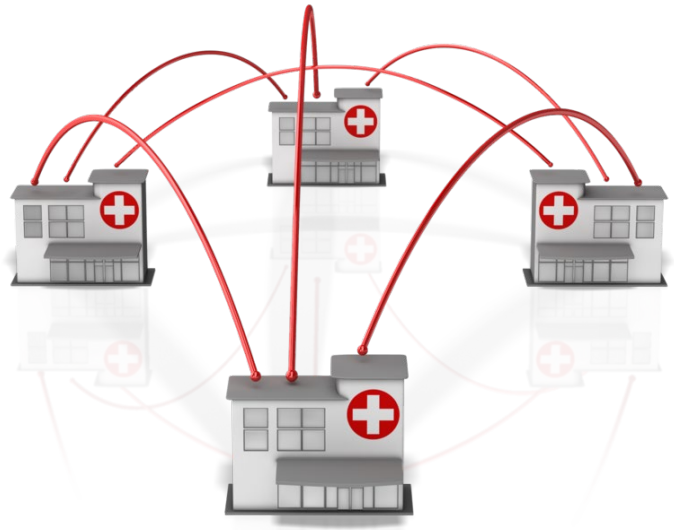


Lessons to be Learned

[Drug development] "responsibility is shared by companies, regulatory authorities, health professionals, and society as a whole."—ICH E11

- Pharma should share development with society and not just "hire it out"
- Science may be exceptional, but may not satisfy regulatory requirements and vice versa
- Academics often have expert knowledge but not infrastructure
  - May not have intimate knowledge of the molecule
- Ownership of data

** https://www.ich.org/fileadmin/Public_Web_Site/ICH_Products/Guidelines/Efficacy/E11/E11_R1_GuidelineWithAddendum_Step4.pdf



CROSS COLLABORATION

EFFICIENT PEDIATRIC TRIAL NETWORKS

Collaboration: an approach to overcome some of the hurdles of pediatric drug development

Realtors versus Builders

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We need to be builders