

Regulating for better treatment and access

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Efficiency

Current paradigm for clinical
development:

phase 1

phase 2

phase 3

Future paradigm:

phase 1

phase 2

phase 3

Interactive drug regulatory processes

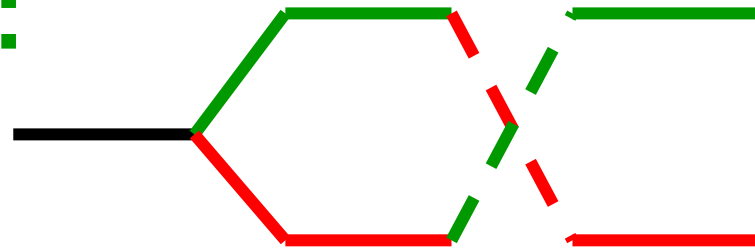
Responsive ethical review

Conventional designs

Parallel groups:



Cross-over:



Alternative designs

RCT variants:

- Pragmatic designs
- Sequential analyses
- Adaptive designs
- Preference designs

Non-randomised controls:

- Explicit historical controls
- Implicit historical controls
- Before and after studies
- Controlled case-series

Case-series:

Conventional analysis

Frequentist:

- Rigid, intellectually questionable, counterintuitive
- Based on the “null” hypothesis
- Type 1 error ($\alpha < 0.05$)
- Type 2 error ($\beta > 0.80$)

Thomas Bayes

No power calculations

No pre-specified sub-groups

No need for stopping rules

Takes account of previous findings

