

Innovative trials

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GOOD
MEDICINES
USED
BETTER

- Increased potential and need for smarter and more efficient trials
- Examples of innovative trials
 - Trials adapted based on interim data
 - Trials augmented with external control data
 - Trials leveraging real-world data
 - Platform studies with a similar protocol and infrastructure
 - Trials using AI to support trial conduct and outcome evaluation
 - Trials using digital tools to capture endpoints remotely
- Key benefits
 - Trials of the right size for reliable evaluation, with fewer patients suboptimally treated
 - A broader population included, with reduced participant burden, at lower cost



Randomized designs with RWD elements: the best of both worlds?

- Combine advantages of randomization with efficiency and capture of real-world use
 - Examples: registry-based trials, pragmatic trials, trials augmented with real-world data
- Key benefits
 - Leverage existing infrastructure and longer term data collection
 - Better capture real-world population and conditions of use in real-world setting
 - Reduced patient burden
- Key challenges
 - Capture of relevant (safety) endpoints
 - Heterogeneous standards of care, irregular timing of visits, data quality
 - Real-world not the same geographically and dynamic



- Innovative trials combine advantage of randomization with efficiency and reduced patient burden, by using innovative methodology and leveraging data external to the trial
- Innovative design elements should be selected carefully to generate the required level of evidence
- To unlock the full potential of innovative trials, more exchange and collaboration is needed
 - ... between trialists, real-world data holders and regulators
 - ... between experts in trial design, causal modelling and artificial intelligence
 - ... with patients

