AusHSI Australian Centre for Health Services Innovation

QUT

Evidence for informing economic evaluations and policy in the digital age: considering advances in trial design, implementation evaluation methods and causal inference

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ACKNOWLEDGEMENT OF TRADITIONAL OWNERS

QUT acknowledges the Turrbal and Yugara, as the First Nations owners of the lands where QUT now stands. We pay respect to their Elders, lores, customs and creation spirits. We recognise that these lands have always been places of teaching, research and learning.

QUT acknowledges the important role Aboriginal and Torres Strait Islander people play within the QUT community.

Health Economic Models and Evidence

Impact of Evidence Quality

Credibility and reliability of health economic models directly influenced by the nature of supporting evidence

Role of Randomized Trials

Randomized controlled trials have long provided high-quality evidence of clinical effectiveness… but…

Uncertainty with Weaker Evidence

Models based on observational studies or expert opinion may have greater uncertainty and limit strength of recommendations… but…

… what about in the ‘Digital Age’

Balancing Rigour and Pragmatism

Importance of Rigour

Methodological rigour ensures scientifically valid results in health economic models, requiring high quality data on effects and resource use.

Value of Pragmatism

Pragmatism enables timely, actionable evidence by relying on simplified data or assumptions, making models practical for decision-making.

Achieving Balance

Balancing rigour and pragmatism makes models credible and relevant, supporting effective policy and clinical decisions.

Increasing Popularity of Hybrid Implementation-Effectiveness

Type 1 Hybrid: Effectiveness First

Primarily evaluates intervention effectiveness, with secondary observation of implementation strategies and context.

Type 2: Balanced Evaluation

Equally assesses both effectiveness of the intervention and the implementation process, providing balanced insights.

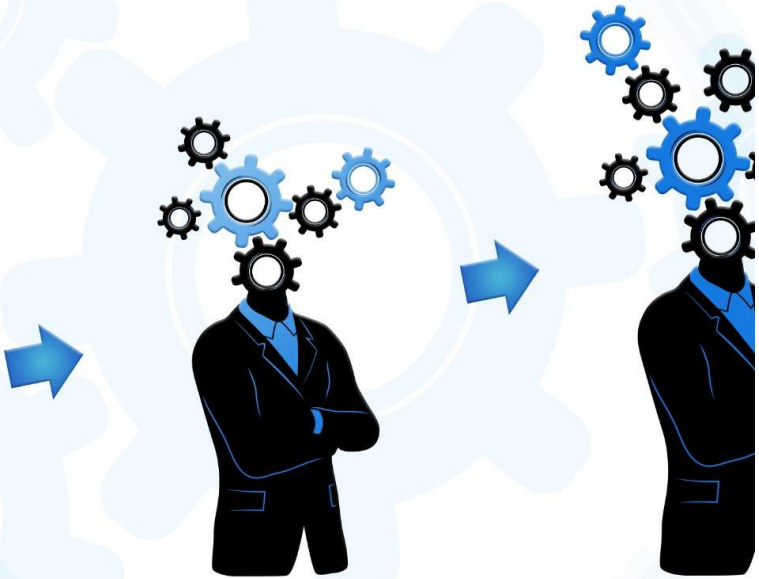
Type 3: Implementation Focus

Focuses on implementation strategies as the main outcome, with effectiveness measured as a secondary outcome.

Use in economic evaluations

All implementation-effectiveness types can be used to inform economic evaluations and can offer rich contextual information (e.g., reach, adoption)

Understanding (full) Implementation Trials



Evaluating Adoption Strategies

Implementation trials assess methods to encourage the uptake of interventions in practical environments, focusing on adoption rather than effectiveness.

Design Variations in Trials

Can use similar designs variations like cluster randomisation, stepped wedges or staircase designs but focus is on implementation outcomes .

Optimising Evidence-Based Integration

The main goal is to facilitate the integration of evidence-based practices, interventions or care models into real-world settings for lasting impact.

Use in economic evaluations

Can inform important economic modelling considerations related to realistic expectations and cost-effective strategies for adoption, scale, and spread.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Digital Maturity Can Improve Evidence… but…

Efficient Real-World Data Collection

Digital maturity can enable efficient collection of real-world healthcare data, complementing traditional clinical trial methods for evidence generation… but…

New Evidence Creation Methods

Digitally mature systems support innovative methodologies for evidence creation, surpassing the capabilities of analogue tools… but…

Advanced Analytics and Data Governance

Strong analytics and appropriately responsive governance are important and interpret complex healthcare data responsibly and efficiently.

Timely Cost-Effectiveness Evaluations

Digital maturity enables prompt assessments of cost-effectiveness using real-world evidence, with potential for improving decision-making in health systems.

Scope and Limitations: What Is Changing

Why it matters for economic evaluations

Strengths

Enhanced data richness and continuous capture has potential to enable deeper insights and more timely evaluations.

Weaknesses

Data heterogeneity and potential biases complicate model adaptation and interpretation.

Opportunities

Adaptive analytic techniques and expanded evidence scope have potential to improve economic evaluations.

Threats

Traditional frameworks may lag behind, risking misinformed resource allocation decisions and policy recommendations.

Innovative Study Designs

Adaptive Trial Flexibility

Adaptive trials have potential to use flexible protocols that adjust in response to new data, speeding up research and improving outcomes.

Pragmatic Study Relevance

Pragmatic studies emphasise real-world applicability, making research findings directly relevant to daily medical practice.

Implementation Science Integration

Embedding implementation science ensures research evidence can be effectively translated into impactful healthcare solutions.

Target Trial Emulation and Real-World Evidence

More robust cost and effect estimates are possible in the absence of traditional clinical trial evidence.

Sequential Multiple Assignment Randomised Trial (SMART) Designs

Multiple Randomisations

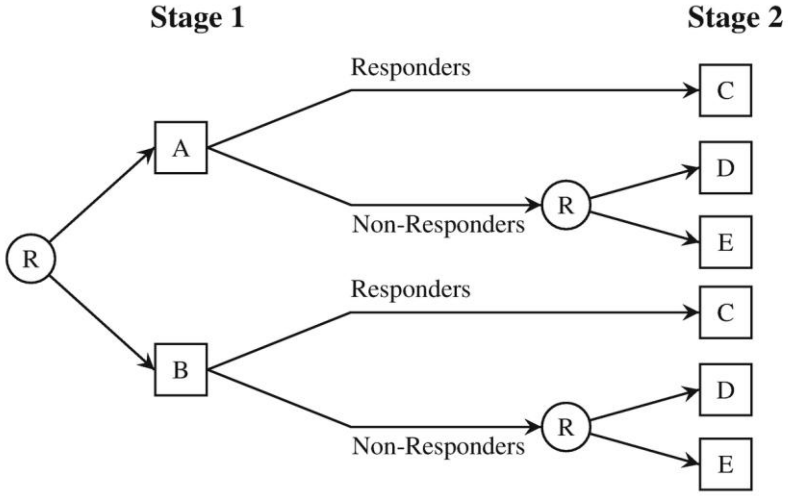
SMART designs allow participants to be randomized at several key points, adapting to their responses and needs.

Reflects Real-World Decisions

SMART designs mimic real-world clinical decision-making by dynamically adjusting treatments and interventions based on patient progress.

Evaluating Personalized Strategies

By comparing tailored treatment strategies, SMART designs provide insights on effectiveness and feasibility for optimizing patient care.



Examples in Chronic Disease and Behavioral Health

Chronic Disease Management

SMART designs enable dynamic adjustments to medication regimens, improving chronic disease treatment effectiveness.

Behavioural Health Interventions

Adaptive therapy frequency in behavioral health helps tailor treatment to individual patient needs for better outcomes.

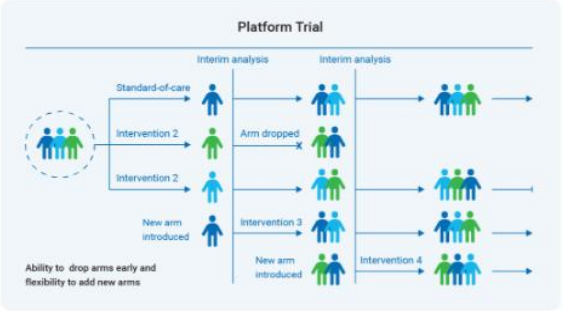
Personalised Treatment Sequences

SMART trials identify optimal, patient-specific treatment sequences to enhance health outcomes across conditions.

Implications for Economic Evaluations

Can require more advanced economic modelling approaches to capture appropriate levels of complexity to inform policy and planning related to personalised approaches

(Adaptive) Platform Trials



Adaptive Trial Design

Adaptive platform trials evaluate multiple treatments simultaneously within one clinical study framework.

Predefined Modification Rules

Trials use predefined rules to adjust patient allocations or add/drop treatment arms based on data.

Core Principles (in theory)

Flexibility, efficiency, and continuous learning support faster effective intervention identification with statistical rigor.

In Practice…

Require extensive planning and up-front work, probably require several comparison arms and/or adaptations to justify additional establishment effort.

Comparison with Traditional Clinical Trial Designs

Traditional Fixed Trials

Evaluate a single intervention at a time with fixed design parameters and no changes during the trial.

Adaptive Platform Trials

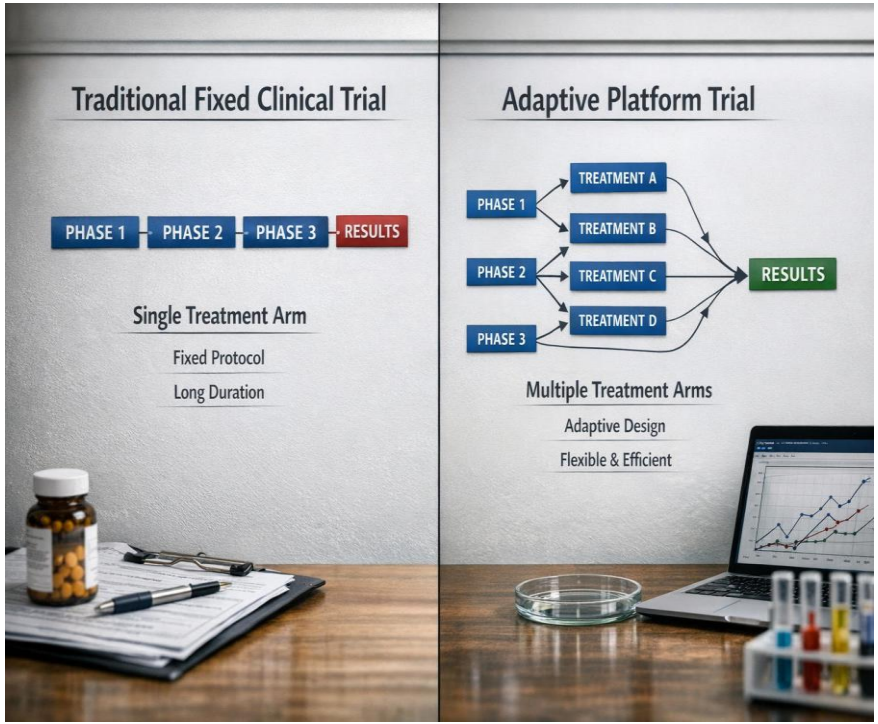
Allow modifications based on interim results, reducing study duration and optimizing resource use.

Concurrent Multiple Interventions

Test multiple interventions concurrently against a shared control group, enhancing efficiency over separate trials.

Implications for Economic Evaluations

Bayesian framework tends to align well with foundational principles underpinning health economics… but greater planning requirements, more advanced modelling requirements and awareness of confounding risks, including usual care drift



Understanding Target Trial Emulation

Mimicking Randomized Trials

Target trial emulation creates observational studies that replicate randomised controlled trials, improving study design and results.

Protocol Specification

Researchers predefine eligibility, interventions, and outcomes to ensure consistency and clarity throughout the study.

Reducing Bias

This approach minimises bias and improves the validity of causal inferences from real-world data derived from digital healthcare systems data.

Implications for Economic Evaluations

Opens new possibilities for high quality real-world evidence to inform both resource use and effectiveness estimates for economic evaluations.

Take home messages

Digital Systems Can Enhance Economic Evaluations

Digitally mature systems boost the ability to gather valuable real-world evidence for economic evaluations to inform healthcare policies and strategic planning.

Innovative Study Designs

New study designs can help assess how healthcare interventions are implemented, providing insights beyond clinical effectiveness to include costs and effects associated with real-world implementation considerations beyond tightly controlled conditions.

Challenges in Evidence Generation

Despite progress, some of the key challenges include ongoing governance, data quality and standards, source bias, and workforce capacity.

Bringing health innovation to life

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