Cost Effectiveness Analysis in Clinical Trials

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The Chief Health Economist Office (CHEO) was set up with three main work functions…

1. Establish and maintain a framework for the use of health economics and cost effectiveness analysis to prioritise and allocate MOH resources

2. Develop models to estimate the marginal value of units of resources to aid MOH in resource prioritisation efforts

3. Analyse the impact of healthcare financing and manpower policies on healthcare utillisation, expenditures and outcomes

Common Ruler to measure across programs

Most Bang-for-the-Buck

How do we influence change?

Defining Cost Effectiveness Analysis (CEA)

CEA is an economic evaluation which compares the costs and health outcomes of new interventions against a baseline.

To allow for comparisons across interventions, the ratio of the additional cost over the health outcomes gained relative to a baseline is calculated for each new intervention. This is commonly referred to as the Incremental Cost Effectiveness Ratio (ICER):

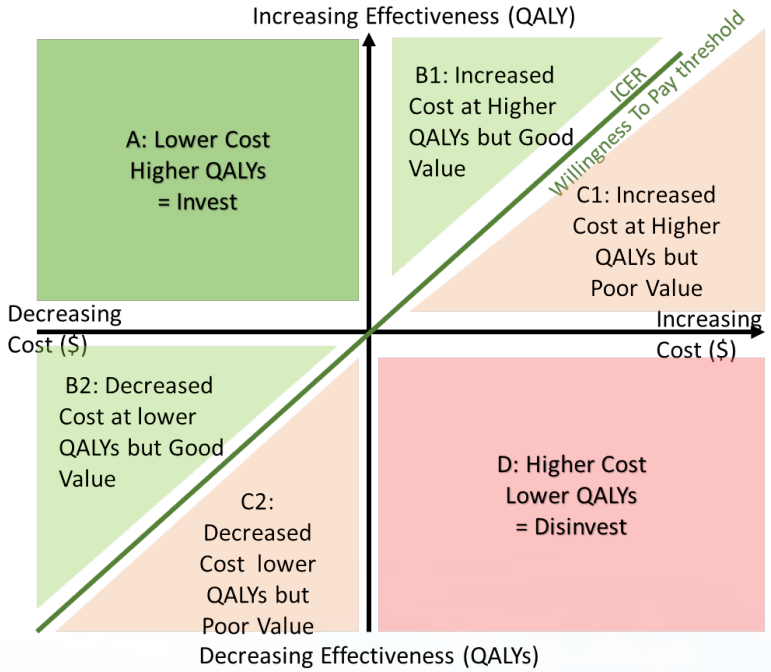
$$ICER = \frac{Cost_{new} - Cost_{baseline}}{Outcome_{new} - Outcome_{baseline}}$$

Health outcomes are measured in standardised units such as Quality Adjusted Life Years (QALYs) to facilitate comparison across interventions

Where resources are limited, economic principles recommend that the marginal dollar be allocated to areas of highest marginal benefits (i.e. most bang-for-the-buck). ICERs allow for comparison across different interventions with the marginal dollar going towards those with the lowest ICERs to maximise the value of our marginal dollar.

Applying CEAs to decision making

A Cost effectiveness plane is typically used to visualise the trade-off between improving health outcomes and higher costs between different interventions:



- The baseline is placed at the origin of the chart and each alternative is positioned based on their incremental QALYs and costs relative to the baseline.
- Most funding requests to MOH will inevitably come from the top-right quadrant (B1 and C1), where improvements in QALYs will come at a higher cost. A decision will have to made on whether these interventions are considered good or poor value based on their ICERs.
- From a cost containment perspective, it is also important to look at alternative interventions in Quadrant B2, which are cost-saving but at the expense of lowering QALYs slightly.

Applying CEAs in MOH and Singapore

Three Key Enablers

to promote and facilitate the use of CEAs for MOH policies and programmes

1 Discipline of applying CEAs in MOH

- This ensures that MOH policy officers consider the transmission mechanisms and impact of their new programmes and policies, and translate them into standardised ICER measures via a CEA.

CHEO's role

- Incorporate CEAs into new programmes and policies.
- Develop a framework for decision-making based on the ICERs generated through the CEAs.

2 Capabilities of key stakeholders

- Development of CEA models is a multidisciplinary process requiring the combined expertise of clinicians, statisticians and model builders.
- Capabilities need to be built up within MOH and eventually, within the clusters.

CHEO's role

- Develop materials and workshops to equip MOH policy officers with the skills to conduct CEAs

3 Synergy with academic institutions

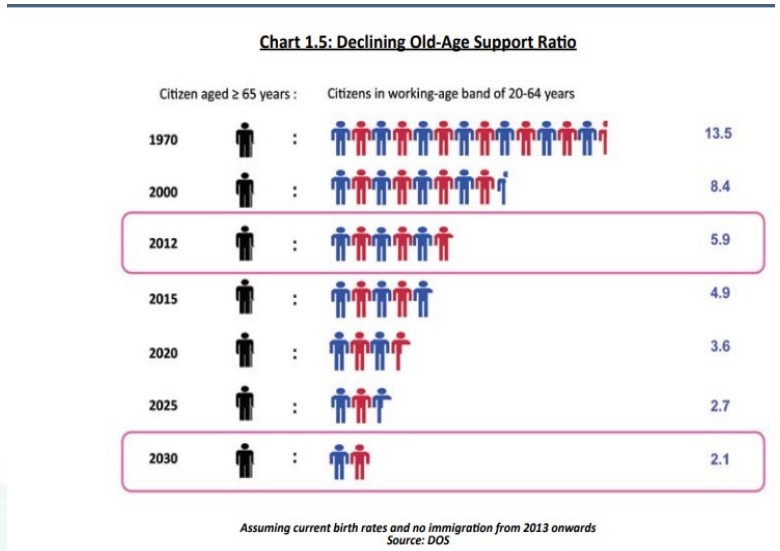
- Collaborations with academic institutions can augment on-going efforts to scale up the use of CEAs in MOH by mitigating the technical limitations and bandwidth constraints within MOH.

CHEO's role

- Engage academics to develop more comprehensive CEA models for complex programmes and policies
- Facilitate admin data access for academics and encourage the building and use of CEA outside of MOHHQ

What is the impetus for the need to prioritise resources on new technology?

The declining old-age support ratio means that we will have less people to 1) paying taxes for healthcare 2) providing care…



- Rising patient expectations
- Limited resources going forward
- Medical technology advancements

Increasingly, policy-makers will demand evidence of economic-value, and cost effectiveness analysis…

Recommendations of good practices in incorporating CEA alongside Clinical Trials have been developed and available

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Cost-Effectiveness Analysis Alongside Clinical Trials II—An ISPOR Good Research Practices Task Force Report

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ABSTRACT

Clinical trials evaluating medicines, medical devices, and procedures now commonly assess the economic value of these interventions. The growing number of prospective clinical/economic trials reflects both widespread interest in economic information for new technologies and the regulatory and reimbursement requirements of many countries that now consider evidence of economic value along with clinical efficacy. As decision makers increasingly demand evidence of economic value for health care interventions, conducting high-quality economic analyses alongside clinical studies is desirable because they broaden the scope of information available on a particular intervention, and can efficiently provide timely information with high internal and, when designed and analyzed properly, reasonable external validity. In 2005, ISPOR published the Good Research Practices for Cost-Effectiveness Analysis Alongside Clinical Trials: The ISPOR RCT-CEA Task Force report. ISPOR initiated an update of the report in 2014 to include the methodological developments over the last 9 years. This report provides updated recommendations reflecting advances in several areas related to trial design, selecting data elements, database design and management, analysis, and reporting of results. Task force members note that trials should be designed to evaluate effectiveness (rather than efficacy) when possible, should include clinical outcome measures, and should obtain health resource use and health state utilities directly from study subjects. Collection of economic data should be fully integrated into the study. An incremental analysis should be conducted with an intention-to-treat approach, complemented by relevant subgroup analyses. Uncertainty should be characterized. Articles should adhere to established standards for reporting results of cost-effectiveness analyses. Economic studies alongside trials are complementary to other evaluations (e.g., modeling studies) as information for decision makers who consider evidence of economic value along with clinical efficacy when making resource allocation decisions.

Keywords: clinical trial, cost-effectiveness, economic, guidelines.

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Typical job of a CEA modeller…

QALYs

- Find literature to map report outcomes to QALYs

Costs

- Find literature that report direct costs
- Impute costs based on reasonableness

Treatment Effects

- Conduct Meta-Analysis to obtain treatment effects

Long Term Outcomes

- Markov model to extrapolate from reported outcomes

How we can collect CEA outcomes as part of clinical trials

QALYs

- Find literature to map report outcomes to QALYs

Collect Quality of Life outcomes (EQ5D-5L, SF6) that can map to Quality of Life weights at appropriate indicator

Costs

- Find literature that report direct costs
- Impute costs based on reasonableness

Leverage on administrative data to collect direct healthcare cost
Supplement with participant surveys for indirect costs

Treatment Effects

- Conduct Meta-Analysis to obtain treatment effects

Directly obtained study, however treatment effects on secondary outcomes can also be captured (e.g. medium-term outcomes)

Long Term Outcomes

- Markov model to extrapolate from reported outcomes

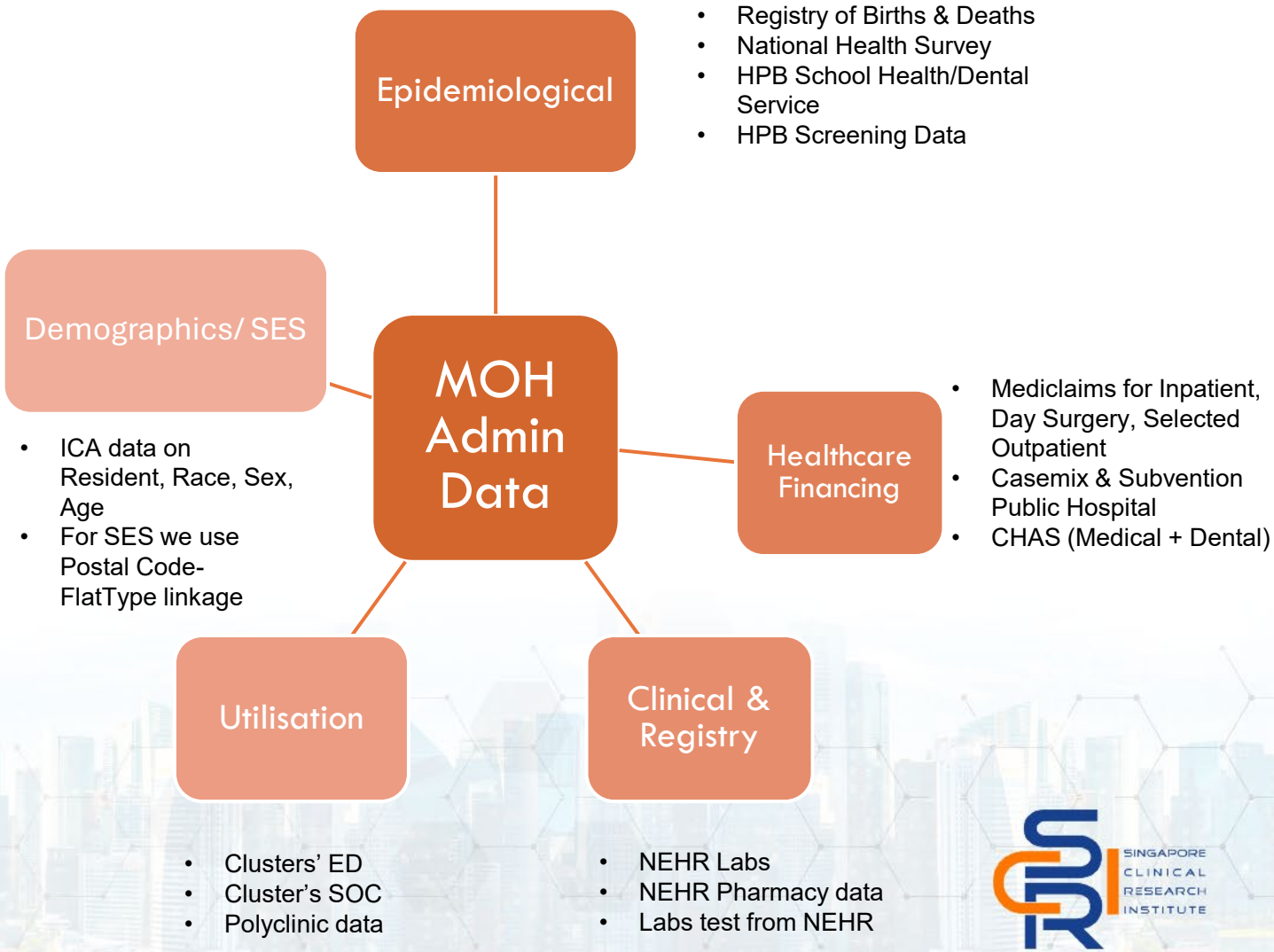
Leverage on administrative data to do long-term tracking of outcomes.

Checklist for Clinical Trials

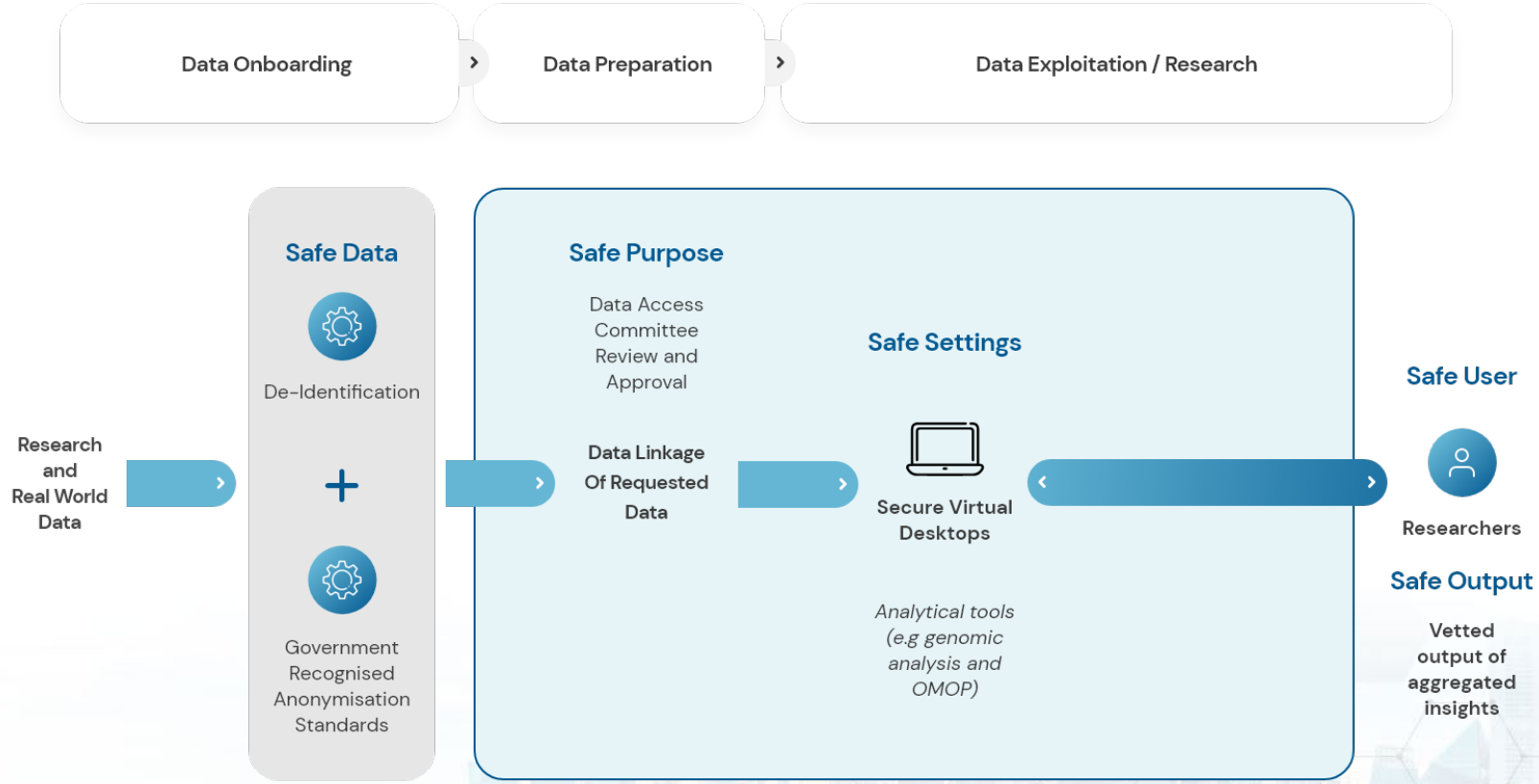
- ❑ Include patient consent clauses for linkage with MOH administrative data
- ❑ Include data-linkage plan as part of IRB
- ❑ Include necessary questions for Quality of Life, Caregiver Burden
- ❑ Include questions on costs not captured by MOH administrative data
- ❑ Start a TRUST data request for data-linkage

TRUST serves to bring together different datasets to enable health-data analytics and research

- National health-data exchange platform to facilitate secure data linkage and analysis of anonymised research and real-world data between the public and private sectors.
- Supports research under the Research, Innovation and Enterprise (RIE) 2025 Human Health & Potential (HHP) domain's focus and priorities.



After data is de-identified and fused, researchers can access it via Secure Virtual Desktop



Thank You!