HTAi HEALTH TECHNOLOGY ASSESSMENT iNTERNATIONAL

Update on the Use of Real-World Data and Real-World Evidence to Support Drug Reimbursement Decision-Making in Asia (The REALISE Project)

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On behalf of the REALISE working group

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Outline of Talk

1) Recap the REALISE Project
2) Update on Project RODEO

REALISE

Duo Meanings

(1) To cause to happen

- Maximize the potential of real-world data (RWD) and real-world evidence (RWE)

(2) To be aware of

- Strengths and limitations of RWD/ RWE

ADVANCING THE USE OF REAL-WORLD DATA & REAL-WORLD EVIDENCE TO SUPPORT DRUG REIMBURSEMENT DECISIONS IN ASIA

Recommendations from the REALISE Working Group*

Reference: ***Use of real-world data and real-world evidence to support drug reimbursement decision-making in Asia.*** *A non-binding guidance document prepared by the* ***REAL*** *World Data* ***I****n A****S****ia for H****E****alth Technology Assessment in Reimbursement (****REALISE****) working group*

Theme 1

When is the use of RWD and RWE appropriate?

When RCTs are lacking and/or the study time frame is insufficient to capture final endpoints

Rare diseases

Localizing established economic models

Re-evaluation of initial reimbursement decisions and price negotiation

Theme 2

What, where and how to collect RWD?

Details, and the pros and cons of each are in the full guidance document

What RWD to collect?

- Population characteristics
- Data on intervention and control
- Treatment effectiveness, safety, and adherence
- Patient-reported outcomes
- Costs

Where can RWD be found?

- Registries
- Claims database
- Electronic medical records
- Surveys
- Wearables and personal tracking devices

How to collect RWD?

- Observational data (cohort, case control, case series)
- Pragmatic clinical trials
- Single-arm studies

Fit-for-use RWD for HTA is a challenge because these data were not originally intended for research. Data quality management and validation protocols should be considered as best practices in data collection.

Key recommendations for improving the process of data collection

Standardize

Standardize the RWD variables

Incentivize

Develop incentives for quality data capture

Balance

Balance patient data privacy protections and access of RWD as public good

Assess

Assess the costs and benefits of data collection

Make credible

Increase the credibility of RWD study designs

Theme 3

From RWD to RWE

Challenges in analyzing RWD

1. Confounding effect
2. Selection bias
3. Missing data

**A concise explanation of methods to address these challenges are detailed in the full guidance document.*

Key procedural recommendations for RWD analysis

- Clearly specify outcomes that are used in analyzing RWD
- Justify the choice of analysis methods used to adjust for confounding and bias
- Where possible, use more than one approach in the analysis
- Be transparent -- publish codes and packages used as appendix

Dissemination Plan

- Public review of the guidance document - completed
- Customized versions of the guidance document for clinicians, regulators and HTA analysts - completed
- Roadshows – completed several; ongoing
- Training workshops – conducted several; ongoing
- Publications
- Translations (Volunteers welcomed!)

REALISE Guidance Document v1.1

SCAN ME

REALISE Country Survey

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Next Step – Project RODEO

Developing a Core Real WOrld Dataset for Economic Evaluations of Oncology Drugs (RODEO)

Funded by National Medical Research Council

HPHSR Clinician Scientist Award

RODEO - Overview

PHASE I

PHASE II

Core Oncology Real-world Dataset

Specific Aim 3: Demonstrate use of Core Oncology Dataset

PHASE III

(A) Comparative effectiveness studies

(B) Electronic phenotyping

Project RODEO

Overall aim:

To curate three core real-world datasets in **lung cancer**, **ovarian cancer** and **multiple myeloma** that will facilitate efficient, accurate, and reliable use of real-world data in health technology assessment (HTA)

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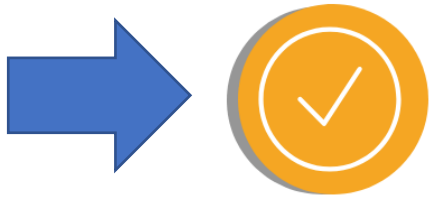
Project RODEO

Objectives:

1. To assess **data quality** by characterizing completeness and nature of missing data from real-world sources
2. To **process unstructured data** using machine learning (ML) algorithms to facilitate data analyses and achieve standardization
3. To **demonstrate the use** of the core real-world dataset through the following studies:
   a) Comparative effectiveness of selected novel therapies
   b) Electronic phenotyping of responders to treatments

Key recommendations for improving the process of data collection



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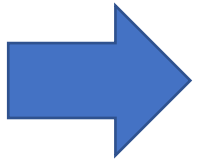

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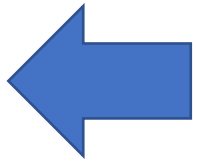

Co-investigators

- **Bioinformatics:**
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- A/P Marcus Ong, SGH
- A/P Shao Wei Lam, SGH

- **Ovarian cancer:**
- A/P David Tan, NUHS

- **Multiple Myeloma:**
- Dr Melissa Ooi, NUH
- Dr Allison Tso, TTSH
- Dr Chandramouli Nagarajan, SGH

- **Lung Cancer:**
- A/P Daniel Tan, SGH/NCCS
- A/P Iain Tan, NCCS
- A/P Ross Soo, NUH

Progress

Collected expert inputs on key data elements (i.e. core dataset)

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Next Steps

1. Sign project agreements with the three sites
2. Text mine ECOG status from case notes
3. Text mine documentation of treatment response from case notes
4. Analyze dispensed medication data for treatment response
5. Compare the congruence of case notes and dispensed medication data as sources of information on treatment response, etc

Potential outputs

- Real-World Patient Characteristics
- Treatment Patterns
- Mutation Testing Patterns
- External control cohorts
- Comparative effectiveness analyses
- Prognostic value of novel biomarkers

Acknowledgement

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- Jeonghoon AHN, Dechen CHOIPHEL, Anne Julienne GENUINO, Anna Melissa GUERRERO, Budi HIDAYAT, Yuehua LIU, Mardiati NADJIB, Ryota NAKAMURA (Theme 3 Advisor), Fiona PEARCE, Shankar PRINJA, Raoh-Fang PWU, Asrul Akmal SHAFIE, Binyan SUI, Auliya SUWANTIKA, Hui-Min WU, Kun ZHAO

- **REALISE International Advisory Panel**
- Amanda ADLER, Kelvin CHAN (Theme 3 Advisor), Brendon KEARNEY, Sean TUNIS, John ZALCBERG

- **REALISE Core Team Members**
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- Wanrudee ISARANUWATCHAI, Yot TEERAWATTANANON, Hwee-Lin WEE (Eds.)

Thank You

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