Use of Real-World Data and Real-World Evidence to Support Drug Reimbursement Decision-Making in Asia

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Outline

- Rationale and Project Aims
- REALISE Guidance document
  - Theme 1: Scenarios to use RWD/ RWE
  - Theme 2: Collecting RWD
  - Theme 3: From RWD to RWE
- Dissemination effort
- Next steps

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Rationale and Project Aims

The majority of clinical trials are conducted outside of Asia and Asian populations are often underrepresented in pivotal clinical trials.

Although there is considerable potential to use RWD to inform HTA decision making, no uniform guidance existed for the Asia region.

The project aimed to develop a non-binding guidance document that will provide a framework to generate and use real-world data (RWD) / real-world evidence (RWE) in a consistent and efficient manner for decision-making in Asia.

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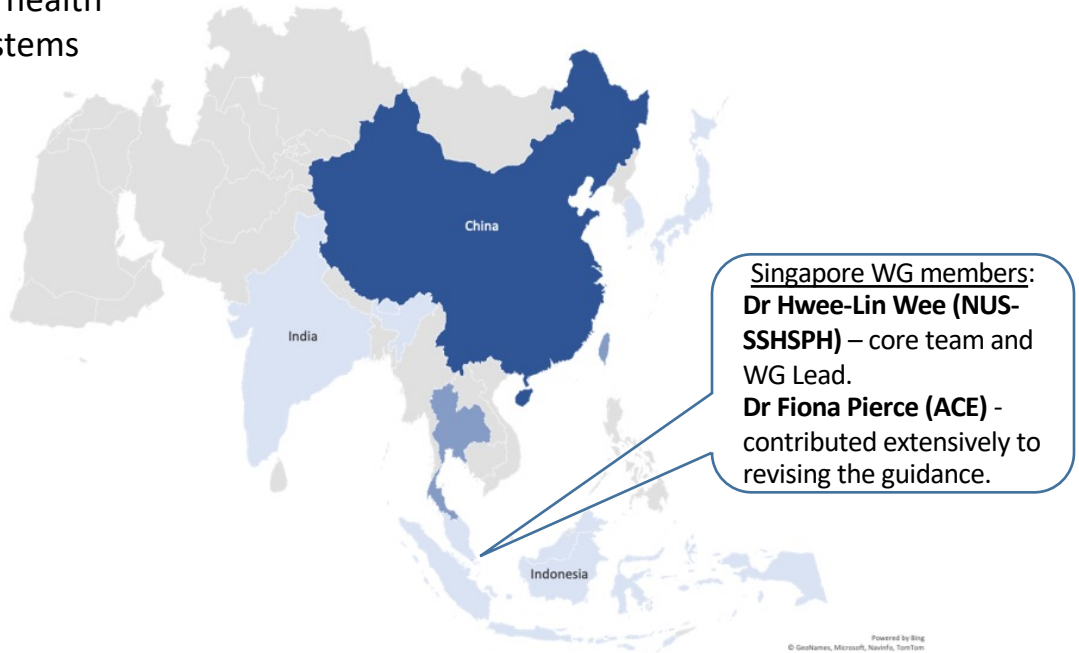
Key Players in REALISE

11 health systems

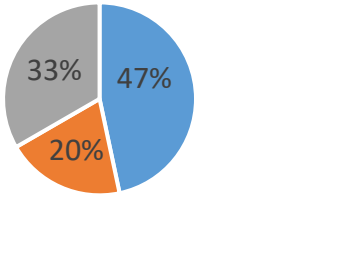


Singapore WG members:
Dr Hwee-Lin Wee (NUS-SSHSPH) – core team and WG Lead.
Dr Fiona Pierce (ACE) - contributed extensively to revising the guidance.

Core team and International Advisory Panel



WG composition



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Data Collection Methods





Online survey

Jointly developed by NUS and HITAP, comprising the following sections:

i) Current practice - use of RWD/RWE for HTA for reimbursement decisions

ii) Current practice -pragmatic clinical trials

iii) Challenges encountered in RWE/RWE generation

iv) Availability of local guidance on RWD/RWE generation

In person meetings

1. April 2019 at 8th HTAsiaLink meeting held in Seoul, Korea

- Consolidated Project REALISE organization
- Country experiences

2. Oct 2019 in Singapore

- Collation of case studies
- Dissemination plan
- In-depth interviews with key informants

Teleconference

Held on 3 June 2019,

Case presentation of how RWE informed a specific policy change in Australia follow by discussion.

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Survey Findings

- All 11 health systems indicated that their HTA agencies accept currently accept clinical effectiveness data from real-world data sources as supplementary evidence to randomized controlled trials (RCT) or where RCTs are lacking

- China, Indonesia, Japan, Malaysia, Singapore, South Korea and Thailand accept data from pragmatic clinical trial as supplementary evidence to randomized controlled trials (RCT) or where RCTs are lacking

FULL TEXT

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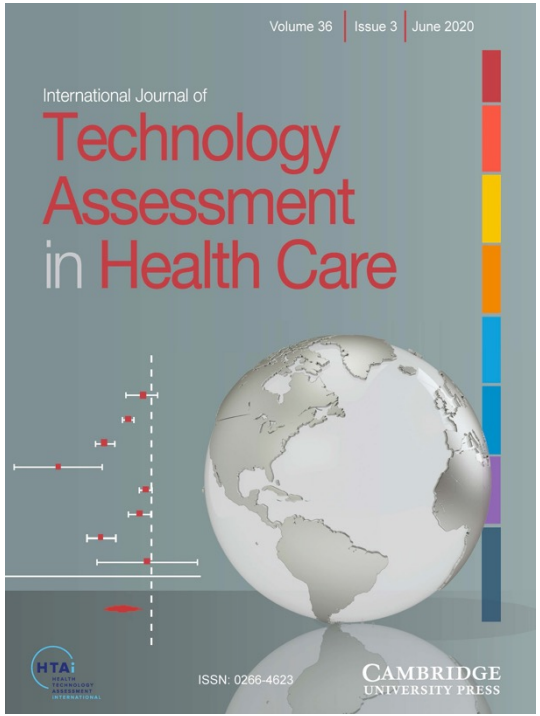
Survey Findings

- Major challenges encountered by HTA Agencies in the use of RWD/ RWE
  - Selection bias
    - Not enough evidence in the HTA dossier that the patient sample selected truly reflects the patients in routine clinical care
  - Confounding
    - The HTA dossier did not clearly report other treatments that the patients are receiving / other comorbidities that the patients have



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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*

Link to REALISE Guidance

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

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Localization of economic models using RWD

Box 2.5. Examples of localization of economic models using RWD (Taiwan and Malaysia)

Taiwan: With a well-developed national database of registry and claims data, Taiwan was able to utilize RWD to localize their studies across diseases. One example is the evaluation of the long-term cost-effectiveness of different cervical cancer screening strategies in Taiwan.[6] Chow et al. used a natural history model for cervical cancer adopted from the literature, and estimated survival rates for cervical cancer over different time horizons from the Taiwan Cancer Registry. Age-specific mortality was obtained from the Department of Statistics for Taiwan's female population; local direct medical costs from the Bureau of National Health Insurance (NHI) and another local publication. With these findings, the authors recommended a screening strategy for combined human papillomavirus (HPV)-Pap smear every 5 years for the publicly financed healthcare system, over the other 8 strategies evaluated.

Malaysia: Epidemiology and resource utilization evidence generated from the Malaysian Dialysis & Transplant Registry was used in an HTA which compared single use vs reusable dialyzers in hemodialysis.[35]

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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.

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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

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Dissemination effort

- Presentations
  - Virtual ISPOR Asia Pacific (14-16 Sep 2020)
  - Virtual ISPOR Europe (16-19 Nov 2020)
  - HTAi Asia Pacific Forum (19 Nov 2020)
  - INAHTA Congress (Feb 2021)
- Translations
- Publications
  - International Journal of Technology Assessment in Health Care
  - Another submitted



International Journal of Technology Assessment in Health Care

cambridge.org/thc

Commentary

Real-world data for health technology assessment for reimbursement decisions in Asia: current landscape and a way forward

Publication describing the REALISE process

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What is next for REALISE?..

A policy brief

In the next 3 months

In the next 4 months

Translation of the guidance to other Asian languages (Chinese translation ongoing)

Guidance document version tailored to supporting reimbursement decision-making in **rare diseases**

In the next 6 months

In the next 12 months

Guidance document version tailored to the **medical device industry**



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Acknowledgement

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- Amanda ADLER, Kelvin CHAN (Theme 3 Advisor), Brendon KEARNEY, Sean TUNIS, John ZALCBERG

- REALISE Core Team Members
- Diana Beatriz S. BAYANI (Theme 3 Lead), Brandon CHUA, Sarin KC (Theme 1 Co-Lead), Lydia Wenxin LIN (Theme 2 Lead), Jing LOU (Theme 1 Co-Lead);
- Wanrudee ISARANUWATCHAI, Yot TEERAWATTANANON, Hwee-Lin WEE (Eds.)

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