WHO webinar series on country pharmaceutical pricing policies

Using real-world data to generate evidence for informing pharmaceutical regulatory and reimbursement decisions
Evidence and country experiences

The webinar will start shortly

Use Q&A window to post questions (not “Chat”)
- “Q&A” to send your questions to the panellists
- “Chat” ONLY when sharing comments or documents with all participants

Please keep all comments respectful and constructive

The session is recorded for viewing on demand
- Slides and recording will be shared after the session
Panellists

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The United States of America

**Claudia Wild**  
Chief Executive Officer  
Austrian Institute for Health Technology Assessment (AIHTA), Austria
Feasibility of Using Real-World Data to Emulate Post-Approval Confirmatory Clinical Trials

WHO Webinar
March 23, 2022

Joseph S. Ross, MD, MHS
Section of General Internal Medicine, School of Medicine
Center for Outcomes Research and Evaluation, Yale-New Haven Hospital
Potential Competing Interests

Research grant funding through Yale from:

– FDA for the Yale-Mayo Clinic Center for Excellence in Regulatory Science and Innovation (CERSI)

– MDIC to support project collaborations as part of the National Evaluation System for health Technologies (NEST)

– Johnson & Johnson, and formerly from Medtronic Inc., for the Yale Open Data Access (YODA) Project

– NIH/NHLBI, AHRQ

– Laura and John Arnold Foundation to support the Collaboration for Research Integrity and Transparency (CRIT)
Meta Analysis
Systematic Review
Randomized Controlled Trials
Cohort Studies
Case Control Studies
Case Series/Case Reports
Background Information / Expert Opinion

Quality of Inform
Premarket studies were limited, postmarket studies were frequently delayed and not consistently completed.

Not going to be able to address each remaining uncertainty through clinical trials – wasn’t true before, less true now...

Opportunities for real-world data

“Using RWE to begin to address these questions is preferable to having no evidence whatsoever.”

- Jarow et. al., *JAMA* 2017;318:703-704.

Major push in U.S. towards real-world data ...

**21ST CENTURY CURES ACT**

**GOALS OF THE LEGISLATION**

**RESEARCH**
- Remove barriers to research collaboration
- Invest in STEM education
- Provide new incentives for the development of rare disease drugs

**GETTING TREATMENTS TO PATIENTS MORE QUICKLY**
- Foster coordination to find cures more quickly
- Modernize clinical trials to increase access to drugs and treatments
- Incorporate patient feedback in drug development and review process

**KEEPING JOBS HERE AT HOME**
- Ensure U.S. remains a global leader in medical innovation, protecting and creating jobs at home
- Encourage development of new medical apps to save lives and create jobs

#CURESatOne

Required FDA to establish a program to evaluate real-world evidence, defined as data regarding the usage, or the potential benefits or risks, of a drug/device derived from sources other than randomized clinical trials.
Typical/Traditional “RWE” of Today

• Advanced observational research / clinical epidemiology to inform product development
  – Disease prevalence, prognosis & treatment adherence
• Generally used for secondary indication approvals for rare diseases or those with well-understood pathophysiology & progression
  – Limited use for initial regulatory approval decisions
• Most commonly: safety surveillance and registry-based medical device studies
Real-World Evidence

• Observational data sources should not be expected to answer the same clinical questions being answered by traditional clinical trials
What % of clinical trials published in high-impact journals in 2017 had:

- Intervention
- Clinical indication
- Enrollment criteria
- Primary end point that would be ascertainable from EHR or claims data?

**Only 15%**

Source: Bartlett et. al., JAMA Network Open 2019;2:e1912869.
Feasibility of Using Real-world Data to Emulate Postapproval Confirmatory Clinical Trials of Therapeutic Agents Granted US Food and Drug Administration Accelerated Approval

Joshua D. Wallach, PhD, MS; Audrey D. Zhang, MD; Joshua J. Skydel, MD; Victoria L. Bartlett, BA; Sanket S. Dhiruva, MD, MHS; Nilay D. Shah, PhD; Joseph S. Ross, MD, MHS

• RWE defined by the context in which the evidence is gathered – in clinical care or home or community settings as opposed to research or academic environments

• Distinction not based on presence or absence of a planned intervention or use of randomization

Source: Sherman et. al., NEJM 2016;375:2293-2297.
Real-World Evidence

• Observational data sources should not be expected to answer the same clinical questions being answered by traditional clinical trials.

“If you want more evidence-based practice, you need more practice-based evidence.”

Aggregating multiple real-world data sources using a patient-centered health-data-sharing platform


Patient Reported Outcome Measures (PROMs)
- Short, post-procedure, mobile-friendly questionnaire emailed twice weekly for 10 total times immediately post-procedure
- Longer, disease-specific, questionnaires emailed at baseline, 1, 4, and 8 weeks post-procedure
- Email reminder instituted during study

Electronic Health Record Data
- Encounters
- Encounter location
- Encounter diagnoses
- Labs and lab results

Pharmacy Data
- Prescription names
- Prescription source
- Formulations and dosages
- National Drug Code (NDC)
- Start and end dates
- Number of refills available
- Prescriber

Personal Digital Device Data
- Activity, including ambulation and sleep (Fitbit)
- Weight (Withings Scale)
- Single Lead ECG (Kardia Mobile)

This is fine, I can see all the evidence I need from here.
The **REALISE** initiative in Asia:
Use of Real-World Data and Real-World Evidence to Support Drug Reimbursement Decision-Making in Asia

Wanrudee Isaranuwatchai, PhD

23 March 2022
Acknowledgement

• REALISE Working Group members
  • 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
  • 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.)
REALISE
Working Group

11 Health Systems
Working Group composition

- 33% HTA agency
- 47% Academia
- 20% MoH
Rationale and Project Aim

• REALISE signifies our desire to realise (‘to cause to happen or to facilitate’) the potential of RWD/RWE while realising (‘being aware of’) their strengths and limitations

• 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 developed 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
Our Journey

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 the 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

Teleconferences

Held on 3 June 2019
Case presentation of how RWE informed a specific policy change in Australia follow by discussion.

And other bilateral / multilateral teleconferences in 2020
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 In ASia for HEalth Technology Assessment in Reimbursement (REALISE*) working group
Theme 1: When
• Scenarios to use RWD/RWE

Theme 2: What
• Collecting RWD

Theme 3: How
• From RWD to RWE
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.
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
2017, we received funding from CIHR’s Partnership for Health Systems Improvement program for the Canadian Real-World Evidence for Value of Cancer Drugs (CanREValue) collaboration. This project is developing a framework for the generation and use of real-world evidence (RWE) for cancer drug funding decisions in Canada.

The CanREValue collaboration brings together key stakeholders including ARCC, CADTH, pCODR, CAPCA, Health Canada, INESSS, Ministries/Departments of Health, pCPA, PMPRB, and provincial cancer agencies from across Canada. By providing a platform for stakeholders to discuss their needs and existing evidence, the CanREValue collaboration will enable

Updated CanREValue Interim Data Report now available!
0 Comments / April 21, 2020

As part of a broader engagement strategy, the CanREValue Collaboration...
Dissemination Efforts

• Presentations
  o ISPOR Asia Pacific (14-16 Sep 2020)
  o ISPOR Europe (16-19 Nov 2020)
  o HTAi Asia Pacific Forum (19 Nov 2020)
  o INAHTA Congress (Feb 2021)
  o HTAsiaLink Conference (Oct 2021)

• Translations

• Publications
  o International Journal of Technology Assessment in Health Care
  o Another submitted

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

Jing Liu1,2,3, Satin KC4,5, Kai Yue Toh2, Saeedmiri Debafi6, Amanda Adler4, Jorgensson Anna4,5, Diane Bertin2, Stephen Chan2,5, Kelvin Chan1,5, Eoorch Chou CS7, Brandon Chu8, Jane Julianne Semper9, Anna Milhous Guerrel10, Breazeal Heaney10, Lycia Wann Leer11, Yuhsueh Liu11, Eiyo Nakamura12, Fiona Pearce13, Shankar Prinja12, Dashiang Peng12, Asrar Ahmad Sardar12, Denny Su12, Aidy Suwantika12, Yeti Setiawati12,13, Srin Tung12,13, Hui Min Wei12,13, Jolin Jabeer12,13, Jun Zhao12,13, Wannidara Saowanawat12,14,15,16 and Hoai Lin Wei12,13

International Journal of Technology Assessment in Health Care

Commentary

"Joint venture - unethical activity to the detriment of the host.

Transcription: Eiyo Nakamura, Asrar Ahmad Sardar, Denny Su, Aidy Suwantika, Yeti Setiawati, Srin Tung, Hui Min Wei, Jolin Jabeer, Jun Zhao, Wannidara Saowanawat, and Hoai Lin Wei
A Call-To-Action

• It is our goal that the proposed guidance document will increase the quality of RWD/RWE collected and used in HTA

• We recognize that the actual implementation of this guidance document will vary from country to country due to many reasons including capacity constraints, lack of political support, and local legislation
(Good) practice organizational models using real-world evidence for public funding of high priced therapies

https://eprints.aihta.at/1329/1/HTA-Projektericht_Nr.138%20.pdf

PD Dr. Claudia Wild, CEO of AIHTA
claudia.wild@aihta.at
Policy (payers) request

RQ1: Which (theoretical) models/ frameworks for setting up such new models for reimbursement with data generation do exist?

RQ2: Of which modules are these models/ frameworks composed/set up? What are their similarities and differences?

RQ3: For which innovative (gene- or regenerative) therapies are these models/ frameworks applied?

RQ4: What experiences are made, and what can be learned from countries further advanced in applying these reimbursement models? What needs to be in place before implementing such models regularly?
Method

- Literature analysis: 16 frameworks identified (4 generic, 12 country-specific models from Belgium, Canada, England, Germany, Italy, the Netherlands, Scotland, and Spain)
- Interviews on Experiences: 11 interviews with 15 experts from eight different countries (Italy, Belgium, Germany, Spain, the Netherlands, Scotland, Canada, Sweden)
- Analysis and synthesis according to modular structure of the models
Results:

Process-modules: Synthesis of essential „ingredients“ of single module along the process

Guidance what to think of in each module
Who initiates the OBMEA and and who selects the technology: only payers? Also patients? or even MAH? - essential elements for commitments, responsibilities, data ownership

- **CAR-T**: initiative of hematologists! (1 database for all patients)
- **Zolgensma**: initiative of payers! (multiple databases for patients)

Feasibility analysis on clear intention and if questions will be answered with which study design:

- Identification of subpopulations that benefit more than others or only access control?
- Infrastructure in place?
- Which outcomes for which decision? Involvement of patients?
Design of data collection based on decisions in step 1

Clear rules on governance:
- Contractual commitments to deliver data
- Contractual agreements on duration of OBMEA and funding of data collection
- Clear responsibilities on monitoring, stopping rules, interim assessments
- Process planning: who does what and when?
- Data ownership!!
Data collection and monitoring:
- Data quality and validity (negative incentives?)
- Combination therapies (Zolgensma + Evrysdi)
- Needs for adaptation of study design

Communication with all stakeholders: patients, parents

Monitoring of markets:
- New competitors, new therapies (haemophilia, CAR-T, SMA-therapies)
Re-Evaluation
• Involvement of stakeholders (patients, parents)
• Fully transparent re-assessment
• Adherence to agreed consequences

Communication
• No closed doors policy!
Exit of OBMEA: Options

- Stopping reimbursement (must be well prepared)
- Reimbursement under conditions (only subpopulations, clear stopping rules, only in specific settings,.....)
- Reimbursement without any further conditions
- Reimbursement under continuous documentation (risk-sharing OBMEA)

Communication of learnings

Dissemination of results

- Facilitation of cross-country learnings through dissemination of results and decisions
- Sharing insights on governance and management issues for future OBMEA, such as separating commercial and performance-related clinical information
- Engagement in pan-European initiatives for future data collections (DARWIN) or interoperable registries and data collections
Learnings and experiences

• Theoretical strengths of OBMEA (reduction of uncertainties) do often not realize in practice
• Very time-consuming, no good data, disinvestment nearly impossible
• Mistrust and scepticism if MAH is collector and owner of data
• Reality: Access under control – not more, not less
• Recommendation: Communication and transparency with all stakeholders is essential
National Health Insurance Reimbursement Policy and RWE in Korea

SUKYEONG KIM PHD
SENIOR RESEARCH FELLOW
NATIONAL EVIDENCE-BASED HEALTHCARE COLLABORATING AGENCY
SEOUL, KOREA
Korea’s National Health Insurance (NHI) is a compulsory social insurance scheme covering 97% of population.

### HTA plays an important role in NHI listing of new health technologies

<table>
<thead>
<tr>
<th>New Health Technologies</th>
<th>Drug</th>
<th>Medical device</th>
<th>Medical procedure</th>
</tr>
</thead>
<tbody>
<tr>
<td>Market approval</td>
<td>MFDS (Safety &amp; efficacy)</td>
<td>MFDS</td>
<td>NECA (nHTA) (Safety &amp; effectiveness)</td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>Company</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Assessment/ Appraisal for NHI listing</td>
<td>HIRA</td>
<td>HIRA</td>
<td>HIRA</td>
</tr>
<tr>
<td>Pricing</td>
<td>NHIS</td>
<td>HIRA</td>
<td>HIRA (RBRV)</td>
</tr>
<tr>
<td>Final decision</td>
<td>NHIPDC</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Notice</td>
<td>MOHW</td>
<td></td>
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</tr>
</tbody>
</table>

NHI reimbursement policy for medicines

- NHI wanted to control rapid introduction of new medicines usually high price/cost
  - All drugs had to apply for listing within 30 days after market approval except drugs used to alleviate minor symptoms (negative list)
  - In 2006, MoHW announced Drug Expenditure Rationalization Plan with introduction of positive list system based on cost-effectiveness, price negotiation, price reduction after patent expiry, and incentives for rational use to control NHI drug expenditure (29% of total NHI expenditure to 24% till 2011)
  - Sponsoring company decides to apply for NHI listing of new drug and submit dossier with evidence of comparative effectiveness and cost effectiveness
  - DBCAC decide recommendations for listing based on the review results of HIRA with maximum allowable price
  - With positive recommendation, price negotiations can be done between company and NHIS, contract can be made on the basis of price and expected volume

Source: PPRI Pharma Profile South Korea, 2018
NHI also introduced special processes because of:

➢ Conflicts from listing failure
  • Drugs which are not cost-effective, show insufficient evidence (orphan drugs), fail price negotiation
  • Recommendation rate was relatively low for anticancer drugs (51.6%) (new drug total: 69.2%)

➢ NHI coverage expansion policy
  • Top priority of health policy to improve low NHI coverage rate (55% in 2011)

➢ Medically important drugs without alternatives in life threatening conditions
  • Indispensable drugs (2007~): drugs for rare conditions, orphan drugs
  • Risk Sharing Agreement (2014~): refund, expenditure cap, limit amount per patient,
  • Economic evaluation exemption (2015~): lowest price suggestion regarding A7 price reference countries
Risk-Sharing Agreements (RSA) expansion & reassessment for drugs

RSA is a system in which the applicant and the insurer share the financial risk and risk of uncertain effectiveness

<table>
<thead>
<tr>
<th>RSA type</th>
<th>Ingredient</th>
<th>Product</th>
</tr>
</thead>
<tbody>
<tr>
<td>Refund</td>
<td>13</td>
<td>26</td>
</tr>
<tr>
<td>Global Expenditure cap</td>
<td>18</td>
<td>29</td>
</tr>
<tr>
<td>Cap per patient use</td>
<td>3</td>
<td>6</td>
</tr>
<tr>
<td>Others (response and refund, etc)</td>
<td>14</td>
<td>24</td>
</tr>
<tr>
<td>Total</td>
<td>48</td>
<td>85</td>
</tr>
</tbody>
</table>

• RSA increased from 14 (2016) to 48 (2020)

• Applied for dupilumab for atopic dermatitis (‘20~) – life-threatening condition?
Trend of pharmaceutical expenditure

- Expenditure reduced in %, but increasing in absolute value

Total % of health spending, 2010-2020

Total, US dollars/capita, 2010-2020

OECD (2022), Pharmaceutical spending (indicator / doi:10.1787/888937852796), accessed on 22 March 2022
Accessibility: NHI listing rate of new drugs

- 65.6%, anticancer drugs 51.6% (~2014) (EY Bae, 2019)
- 67.2%, anticancer drugs 70.2% (2013~2019) (S Park, 2020)
- More than 90% of anticancer drugs approved 2016~2017 were listed through RSA
  - RSA is becoming general process for high-cost anticancer drugs NHI decision making
  - 36% of new anticancer drug expenditure was RSA drugs
NHI coverage improvement is a top priority for the government

- Plan for NHI coverage rate improvement
  ✓ 62.7% ('17) → 70.0% ('22) → 70.0% ('23)

Government policies to improve coverage

- Selective, preliminary coverage with tiered coverage rate
  - Procedures and therapeutic materials
  - Reimbursement rate 50%, 20%, 10%
  - For evidence generation (3~5 years)
  - Assessment for following decision making (NECA - HIRA)
  - 170 items including TAVI, Navigation procedure for surgeries, etc

- Fast uptake for diagnosis with in-vitro diagnostics in nHTA
  - Assessment after entry
  - nHTA suspension for 1~5 years
  - Managed with preliminary coverage of NHI (10%)
  - Assessment with RWE

- Early dialogue for supporting companies preparing better evidence
  - One-stop service for medical device companies from MFDS, NECA and HIRA
NHI policy and RWE

- NHI coverage improvement is top priority of government
  - Government R&D programs for comparative effectiveness research and outcomes research
    - Supporting local evidence generation
    - Supporting clinical, NHI decision making through evidence and guidelines development
  - Ultra high price/cost drugs are approaching with financial and clinical uncertainty
    - Managed entry schemes are needed
    - Reassessment of existing drugs are needed
    - RSA is becoming popular and legal process for evidence generation through real world use – CAR-T has been recommended NHI listing by DBCAC through Global Expenditure Cap + patient outcome-based RSA

- Under COVID19, conditional approval of vaccines and drugs needs RWE
  - PMS using RWD/RWE is under the discussion
    - Supporting CDM researches for PMS
  - Discussion of networking between MFDS and NHIS for RWD/RWE
  - Close relationship for RWE among regulatory and NHI coverage decision making
Challenges

- **NHI sustainability**
  - NHI expenditure is rapidly increasing
    - Korea is the most rapid aging country with the lowest birth rate in the world (0.84, 2020)
  - NHI coverage expansion policy is still in high priority
    - Low coverage rate of public program including NHI
    - Ultra high-cost technologies with high uncertainty are approaching and high needs of NHI coverage from patients and companies
    - NHI’s negotiation power is limited

- **HTA needs are high because of NHI coverage expansion policy in Korea**
  - Increasing needs of evidence generation design and assessment from selective & preliminary coverage policy, conditional use of medical devices and procedures
  - Started outcome-based RSA for ultra high-cost drugs with clinical uncertainty
  - Reassessment of NHI drugs started
Challenges

- **NHI claims data have limited information**
  - Utilization oriented information and insufficient clinical and outcomes information
  - Medical record use is limited because of privacy protection

- **Quality of RWE is limited**
  - Good quality of study design to control compounding factors
  - Pre-determined protocols for good and consistent decision making
  - Clinical study infrastructure including clinician researchers, epidemiologists, data specialists, and education & training programs

- **International cooperation for improving HTA capacity, data & information sharing**
  - Uptake needs for high-cost technologies are global trend
  - Because of global RSA increasing, price information is limited
  - Cooperation for effectiveness assessment, patient registries for rare diseases...
  - Supporting HTA capacity building and experience sharing
Thank You!
sukyeong.kim@neca.re.kr
# National Health Insurance

+ Compulsory social insurance governed by Ministry of Health and Welfare covering 97% of population
+ All medical institutions and pharmacies are obliged to provide services covered by NHI
+ The majority of health care services are provided by private health care institutions, while public health care institutions provide a small range of services.

## Fee-for-service payment
- Outpatient & inpatient services
- DRGs for 7 simple surgeries (mandatory)
- New DRG for public hospitals and voluntarily participating hospitals
- Per-diem for sub-acute & long-term care hospitals

## Copayment at the POS
- 30% for outpatient service & prescription
- 20% for inpatient service
- 5% for cancer patients & CV diseases, serious burn/trauma
- 10% for rare diseases, dementia
- 0% for tuberculosis

## Low NHI coverage
- 62.7% of total health care expenditure in 2017
- NHI coverage expansion has been the top priority of governmental health care policy
- Selective and preliminary coverage policy for evidence development
NHI system

+ MoHW directs and supervises policy measures related to the NHI determining contributions and benefits.
+ NHIS manages the eligibility of NHI beneficiaries, imposes and collects contributions, pays to providers after review, and negotiates fee schedules with representatives of providers, and negotiates the prices of new medicines with pharmaceutical companies.
+ HIRA reviews claims submitted by providers, manages quality assessment programs of services, reviews dossiers submitted by pharmaceutical companies for NHI listing and makes recommendations for medicines, medical devices, and medical procedures and surgeries through relevant committees.

Source: PPRI Pharma Profile South Korea, 2018
Panel discussion
In your view, what are (or what should be) the main purposes of using evidence generated from RWD?

Using RWD to inform healthcare decision making is not new.

- How have the purposes *changed* over time or not?
- How have the purposes been *achieved* or not?
- What are the *benefit-risk trade-offs* in using RWD and RWE? And is it worth it?

Should *financial risk* be part of the benefit-risk equation?
How can RWD and RWE be used in decision making without lowering the evidentiary standards?

What are the main challenges in translating RWD to generate robust RWE?

- data quality, study design and analysis, ethics and other consideration

How could we prevent data ‘torturing’/excessive data dredging?

“If you torture the data long enough, it will confess to anything”

Ronald Coase
Will evidence from RWD replace evidence from (late-phase) clinical trials?

- What would be the **implications** for regulatory licensing, pricing, reimbursement and safe use?

- Should there be **greater standardization and transparency** of evidentiary requirements in regulatory and payer contexts?

  Could there be a **common approach** of evidentiary basis given the need for RWE arose, at least partially, from the need for reflecting RW contexts?
To what extent should there be a reduction in the price of medicines approved based on RWE?

Generating evidence through RWD is considered as more pragmatic and efficient. How much should the public/government be asking for lower price in return for granting earlier market access through ‘lower’ (pre-and post-registration) evidentiary requirements?

“on average, list prices are 5.9% higher if a financial-based or performance-based managed entry agreement exists.

Gamba, Pertile and Vogler. Health Economics, 2020
Post-registration, pricing and reimbursement decisions

- Will companies follow through their pre-registration commitments for gathering RWD and submitting RWE?
- To what extent has real-world evidence been able to mitigate pre-registration uncertainty for regulatory and pricing purposes?

Source: https://www.fda.gov/media/151146/download
Post-registration, pricing and reimbursement decisions

- What (should/would) happen if subsequent data do not confirm the effect from earlier clinical trials?

- How should the system prevent companies from pursuing early market entry through RWE, then expand the market through promoting non-approved ‘off label’ use?

- Change in reimbursement status/rate
- Compensation (financial)
- Liability

E.g. In France, registry data shows that disease progression continued in patients with late-onset Pompe disease who were treated with alglucosidase alpha. This review resulted in **downgrading of rating and reimbursement rate** in 2017.

Q&A with the audience
April webinar

TBC

Where to ask your burning questions?

Comments and suggestions

fairpricing@who.int