RWE4Decisions Case Study Workshops – Key Learnings

10 September 2020

What is RWE4Decisions?

VISION

Stakeholders agree **what** real-world data (RWD) can be collected for highly innovative technologies
– **when**, **by whom** and **how** –
in order to generate real-world evidence (RWE) that informs decisions by healthcare systems, clinicians and patients

KEY PRINCIPLES

RWE4Decisions REAL WORLD EVIDENCE

Role of RWE

Highly innovative technologies are often developed for ultra-rare diseases.

It is recognized that regulators are increasingly authorizing products on the basis of uncontrolled trials.

HTA/Payers still prefer RCTs to demonstrate efficacy, but recognise that when uncontrolled trials are used, RWE will be an important part of the evidence submission, for example to create an external control arm to determine added clinical benefit.

RWE may have a role in resolving uncertainties that can't be resolved by clinical trials, but it is not meant to be a cheap substitution for a clinical study.

INAMI-led initiatives

Objective

To identify evidence gaps that will remain after clinical trials and are likely to be important uncertainties for HTA/Payers and may be resolved by RWE.

TRUST4RD

Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases

Iterative discussions between stakeholders and continuous evidence generation support informed rational evidence generation.

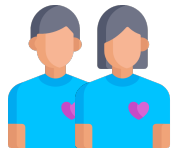
Characterization of uncertainties in terms of natural history of disease, comparator, effectiveness and health system issues.

REAL WORLD EVIDENCE RWE4Decisions

A multi-stakeholder initiative which brings stakeholders together to agree what real-world data could be collected for highly innovative technologies – when, by whom and how – in order to generate real world evidence that informs decisions by healthcare systems (HTA/payers), clinicians and patients.

RWE4Decisions Case Study Workshop (Sept 2020)

Demonstration project of a light-touch, international, multi-stakeholder scientific consultation meetings about development and use of real-world evidence, based on TRUST4RD recommended process
3 highly innovative technologies in development, discussed individually ⇒ confidential advice to inform evidence generation plans and public learnings across discussions.



Patient groups

Payers/HTA bodies (European & national)

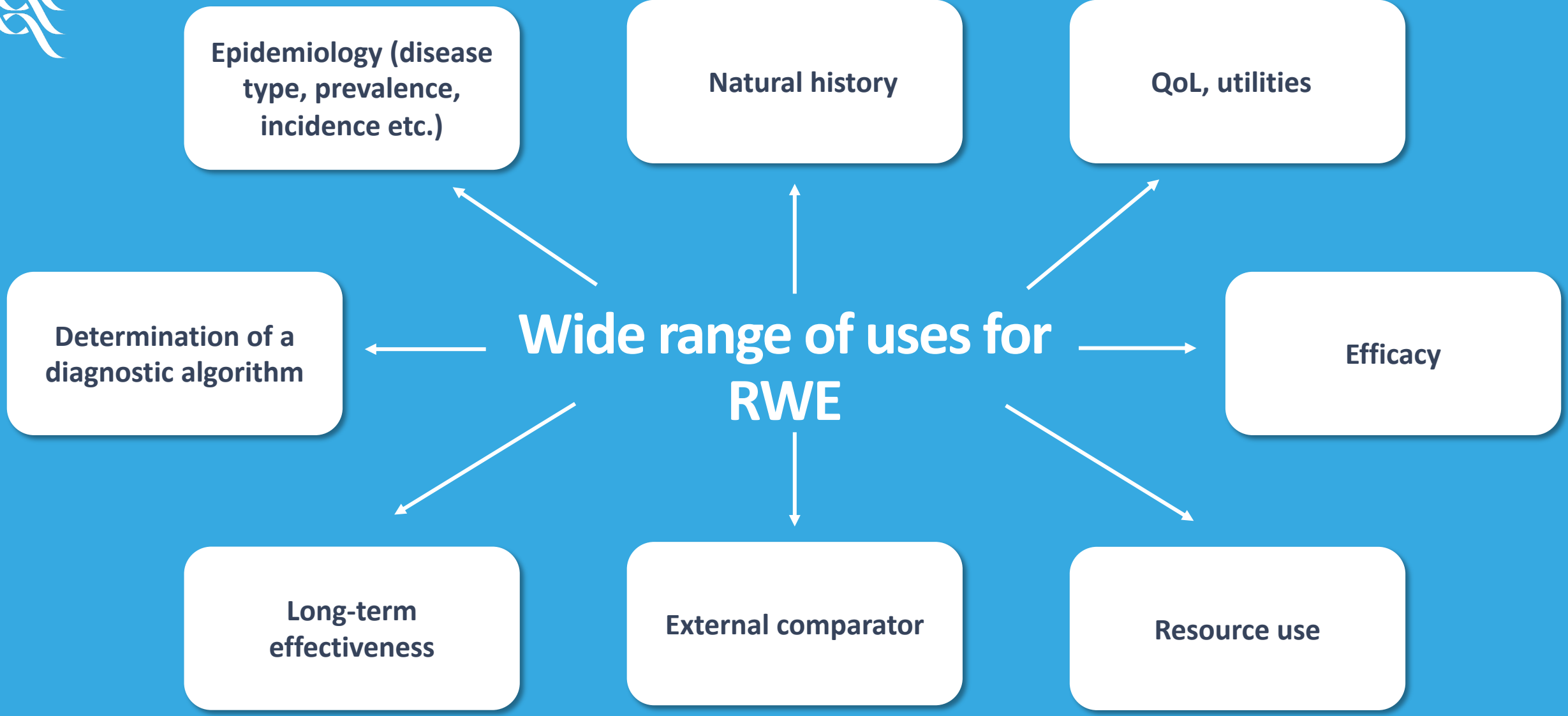
Pharmaceutical Industry

Learn by doing

Registry-holders

Clinicians

INAMI-RIZIV


Epidemiology (disease type, prevalence, incidence etc.)
Natural history
QoL, utilities
Determination of a diagnostic algorithm
Wide range of uses for RWE
Efficacy
Long-term effectiveness
External comparator
Resource use

Workshop outcomes

Honest discussion of RWE generation plans

Consideration of

1. Challenges
2. Pros and cons of different RWE designs/data sources
3. Propositions for long-term evidence generation post-launch
4. Practicalities of data collection (responsibility & approaches to reduce duplication/maximize use)
5. Be clear that RWE may not resolve important uncertainties

Plan iterative dialogues

RWE4Decisions proposes iterative dialogues to discuss RWE requirements

HTA/Payers need to agree which questions should be discussed when (create a timeline) - Link to life cycle of RWD availability

Use of RWE studies

RWE studies to create matched external controls should have clear protocols and analytical plans with robust data capture (e.g. via eCRF) and quality control mechanisms, including analyses to show sensitivity of the cohort to different data rules.

Applicability of RWE studies to different jurisdictions should be considered.

A plan for the use of RWE studies should be developed that does not cherry pick elements.

Recommendations

International disease based registries

Recommended – but practicalities of use for an individual product is complex and needs further discussion (content, funding, management, ownership).

Collaboration across EU

Engage with ERNs and EJPRD to ensure HTA/Payer needs are understood when disease registries are developed and to ensure Payers can have access to relevant data.

Payers

Be clear about what data is required post-HTA/reimbursement and collaborate to define a layered core dataset outlining data that is

- essential
- important
- nice to have

Other initiatives

Avoid duplication with other initiatives (Early Dialogues, etc). Identify purpose of a light-touch process focussed on RWE and where it can contribute given insufficient capacity in other systems.

RWE4Decisions Transparency

- Use clear processes for managing conflicts of interest among stakeholders
  - ***Even "light touch processes" or "demonstration projects" need to be carefully managed when confidential information from drug development is involved***

- Clarify what questions RWD may be able to address in regulatory and Payer/HTA decisions
  - ***Challenges faced by industry***
  - ***Clarify decision-maker requirements (agreements and diversity)***

- *Publish methods for critical assessment of RWE*
- *Share information about RWD studies underway across different jurisdictions to enable data amalgamation*

RWE4Decisions Collaboration

- Iterative dialogues should involve all stakeholders throughout the lifecycle of a technology to discuss plans for evidence generation and the potential for RWE to resolve important decision uncertainties
  - ***Who should organise the dialogues?***
  - ***What questions and stakeholders at what point?***

- RWE generation is a shared responsibility & should be pre-specified & planned with all stakeholders
  - ***How do we identify what really matters to each stakeholder?***
  - ***How can we agree a common core dataset for an international data collection initiative and support appropriate data access across borders?***
  - ***Who pays, who owns, who can analyse?***

RWE4Decisions Collaboration

Each stakeholder needs to take responsibility
for aspects they can influence
and work collaboratively with other stakeholders
to achieve the common goal
of developing RWE that can inform (Payer/HTA)
healthcare decisions and improve patient care