Leveraging Patient-Provided Information to Improve Real-World Evidence

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Workshop

Purpose
• Engage workshop participants on the challenges, opportunities, and methods for leveraging PPI to improve RWE

Overview
• ISPOR-ISPE Task Force Recommendation
• Patient-Centered RWE
• Experience and Suggestions
• Exercise
Speakers

Richard J. Willke, PhD, Chief Science Officer, ISPOR

Cristina Masseria, MSc PhD, Methods & Capabilities Lead, PHI, Pfizer

Chris L. Pashos, PhD, Vice President, Global Evidence Strategy, AbbVie US LLC
ISPOR/ISPE RWE Task Force

Recommendation for Patient Engagement in RWD Studies

Richard J. Willke, PhD, CSO, ISPOR
Read the freely available Good Practices Reports

ispor.org/RWEinHealthcare Decisions
Transparency of study processes

Original Report
Good Practices for Future Comparative Effectiveness Research: The ISPOR-ISTE Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making

PDS Pharmacoeconomics & Drug Safety
Original Report
Reporting to Improve Reproducibility and Facilitate Validity Assessment for Healthcare Database Studies V1.0
Shirley V. Wang1,2,† | Sebastian Schneeweiss1,2 | Marc L. Berger3 | Jeffrey Brown4 | Frank de Vries5 | Ian Douglas6 | Joshua J. Gagne1,2,† | Rosa Gini7 | Olaf Klungel8 | C. Daniel Mullins9 | Michael D. Nguyen10 | Jeremy A. Rassen11 | Liam Smeeth6 | Miriam Sturkenboom12

on behalf of the joint ISPE-ISPOR Special Task Force on Real World Evidence in Health Care Decision Making
Transparency of study processes

Reproducibility of study implementation
Transparency - Primary Recommendations

1. A priori, determine and declare that study is a “HETE” or “exploratory” study

2. Post a HETE study protocol and analysis plan on a public study registration site prior to conducting the study analysis.

3. Publish HETE study results with attestation to conformance and/or deviation from original analysis plan.

4. Enable opportunities for replication of HETE studies whenever feasible (ie, for other researchers to be able to reproduce the same findings using the same data set and analytic approach).

5. Perform HETE studies on a different data source and population than the one used to generate the hypotheses to be tested, unless it is not feasible.

6. Authors of the original study should work to publicly address methodological criticisms of their study once it is published.

7. Include key stakeholders (eg, patients, caregivers, clinicians, clinical administrators, HTA/payers, regulators, and manufacturers) in designing, conducting, and disseminating the research.
Rationale for 7th Recommendation

“The best way to involve stakeholders is to be clear about the intent of stakeholder engagement ...”

“The specific consultative needs will depend on the intended use of the study, endpoints involved, novelty of the approach, perceived reliability of the data, and other factors.”

“Be the ball”
Patient-Centered RWE
Intersection of FDA Initiatives

“Systematic approach to help ensure that patients’ experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.”

“Assist developers interested in using real-world data (RWD) to develop RWE to support Agency regulatory decisions.”

This is not just about PROs

All information regarding a patient regardless of source

A range of input or data that is collected from the patient

Patient Information

Patient-Provided Information (PPI)

Patient-Reported Information

• Patient Reported Outcomes
• Patient Preference Information
• Patient Perspective Information

Patient-Generated Health Data

A subset of PPI that is reported directly by a patient without amendment or interpretation by a clinician, researcher, or any other entity

A subset of PPI that is produced (i.e., created, recorded, or gathered) by the patient or caregiver

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Patient-Centered RWE with Traditional Datasets

How do we apply PPI to study designs that rely on traditional data sets?
Poll

How have you involved patients in RWD studies?

1. I’ve never done a RWD study

2. I’ve never directly involved patients in primary data collection or study design for a RWD study

3. I’ve only collected structured PRO, preference, or economic information from patients in a RWD study

4. I’ve collected less structured information about the disease and treatment experience from patients

5. I’ve involved patients in the design of a RWD study
NHC RWE Research Design Framework

Refined research question

Research protocol

Translation

Lit Review
Preliminary Research Question
Data Availability
Gaps in Knowledge/Rationale
Hypothesis
Target Patient Population

Research Protocol

Data source
Inclusion and Exclusion
Subgroups
Study Design
Covariates
Outcome of Interest/Endpoint

Translation

Hypothesis Generation
Next steps/Promoting uptake
Impact Assessment
Dissemination
Limitations