Leveraging Patient-Provided Information to Improve Real-World Evidence

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MAY 21, 2019


American Heart Association
Learn and Live
American Diabetes Association
Cure • Care • Commitment
National Alopecia Areata Foundation
American Kidney Fund
FIRST
Foundation for Ichthyosis & Related Skin Types
Educate • Inspire • Connect
COPD FOUNDATION
ALPHA-1 FOUNDATION
NATIONAL PSORIASIS FOUNDATION
American Autoimmune Related Diseases Association, Inc.
Huntington's Disease Society of America
PKD FOUNDATION
Polycystic Kidney Disease
Tourette Association of America
Awareness. Research. Support.
NATIONAL Eczema ASSOCIATION
alzheimer's association
AMERICAN LIVER FOUNDATION
Your Liver. Your Life
amputee coalition
THE MARFAN FOUNDATION
Know the signs. Fight for victory.
resolve
THE NATIONAL INFERTILITY ASSOCIATION
colorectal cancer alliance
aafa Asthma and Allergy Foundation of America
MGFA MYASTHENIA GRAVIS FOUNDATION OF AMERICA, INC
PUTTING PATIENTS FIRST
NATIONAL HEALTH COUNCIL
IDF Immune Deficiency Foundation
Arthritis Foundation
Champion of Yes
National Foundation for Ectodermal Dysplasias
American Cancer Society
Women Heart
The National Coalition for Women with Heart Disease
Celiac Disease FOUNDATION
Sjögren's Syndrome Foundation
BEYOND CELIAC
awareness • advocacy • action
NATIONAL OSTEOPOROSIS FOUNDATION
ALS ASSOCIATION
nfed
Supporting you. Supporting each other.
NATIONAL FOUNDATION FOR ECTODERMAL DYSPLASIAS
Barth Syndrome Foundation
MS National Multiple Sclerosis Society
Prevent Blindness
Our Vision Is Visio
Spina Bifida Association of America
OSTEOGENESIS IMPERFECTA FOUNDATION
Unbreakable Spirit
American Foundation for Suicide Prevention
The LAM Foundation
A BREATH OF HOPE
prevent cancer FOUNDATION
Mended Hearts
THE MYOSITIS ASSOCIATION
Hydrocephalus Association
NATIONAL HEMOPHILIA FOUNDATION
for all bleeding and clotting disorders
National Blood Clot Alliance
Stop The Clot®
LUPUS FOUNDATION OF AMERICA
LUNG CANCER ALLIANCE
Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE
NORD
National Organization for Rare Disorders
CMTA
Charcot-Marie-Tooth Association

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

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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

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ISPOR

Improving healthcare decisions

ISPOR/ISPE RWE Task Force

Recommendation for Patient Engagement in RWD Studies

Richard J. Willke, PhD, CSO, ISPOR

VALUE IN HEALTH 20 (2017) 1003–1008

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval

ELSEVIER

Original Report

Good Practices for Real-World Data Studies of Treatment and/or Comparative Effectiveness: Recommendations from the Joint ISPOR-ISPE Special Task Force on Real-World Evidence in Health Care Decision Making

Marc L. Berger[1,*], Harold Sox[2], Richard J. Willke[3], Diana L. Brixner[4], Hans-Georg Eichler[5], Wim Goettsch[6], David Madigan[7], Amr Makady[6], Sebastian Schneeweiss[8], Rosanna Tarricone[9], Shirley V. Wang[8], John Watkins[10], C. Daniel Mullins[11]

Read the freely available Good Practices Reports

ispor.org/RWEinHealthcareDecisions

PDS Pharmacoepidemiology & Drug Safety

Official Journal of the International Society for Pharmacoepidemiology

ISPE

ORIGINAL REPORT

Reporting to Improve Reproducibility and Facilitate Validity Assessment for Healthcare Database Studies V1.0

Shirley V. Wang[1,2] | Sebastian Schneeweiss[1,2] | Marc L. Berger[3] | Jeffrey Brown[4] | Frank de Vries[5] | Ian Douglas[6] | Joshua J. Gagne[1,2] | Rosa Gini[7] | Olaf Klungel[8] | C. Daniel Mullins[9] | Michael D. Nguyen[10] | Jeremy A. Rassen[11] | Liam Smeeth[6] | Miriam Sturkenboom[12] |

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Transparency of study processes

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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

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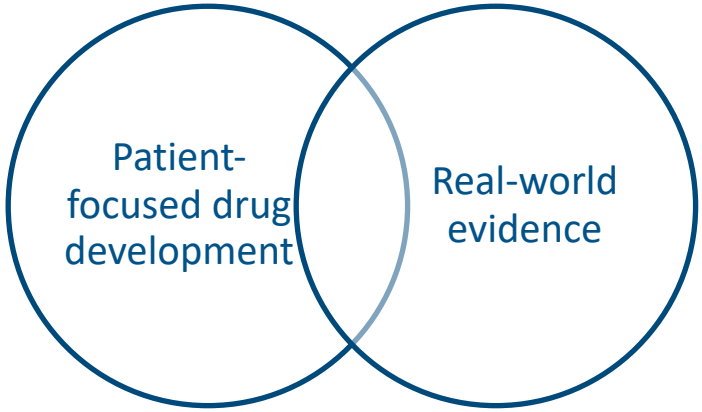
FDA. Patient-Focused Drug Development. https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

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."

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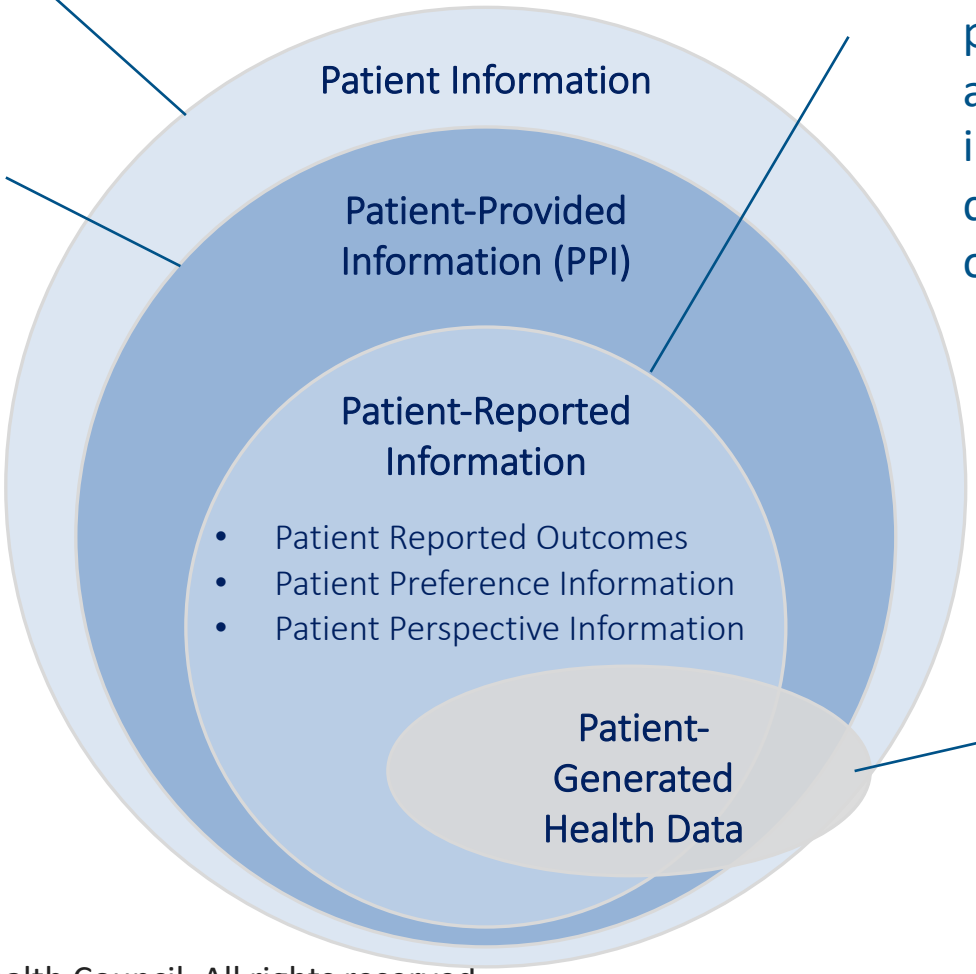
FDA. Patient-Focused Drug Development. https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

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



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

Patient Engagement → PPI → ?

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

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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

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NHC RWE Research Design Framework

Refined research question

Research protocol

Translation

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