PATIENT-CENTERED METHODS: ACHIEVEMENTS, INNOVATIONS, AND THE FUTURE

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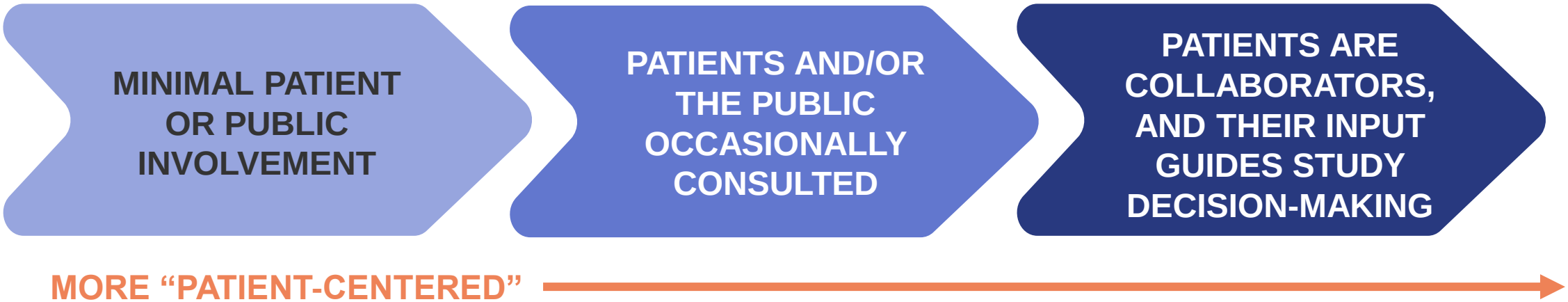
What is Patient Engagement in Research?

"The active, meaningful, and collaborative interaction between patients and researchers across all stages of the research process, where research **decision making is guided by patients**' contributions as partners, recognizing their specific experiences, values, and expertise."

- ISPOR definition



Source: Harrington RL, Hanna ML, Oehrlein EM, et al. Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group. *Value Health*. 2020;23(6):677-688. doi:10.1016/j.jval.2020.01.019

What is in our (continuously evolving) toolbox?

RESEARCH METHODS

PATIENT ENGAGEMENT

- Engage patients to learn from patients and caregivers about experiences, priorities, and needs
- Engage members of the patient community to apply what they already know about patient experiences, priorities, and needs to improve data collection or another activity

PATIENT-CENTERED RESEARCH

- Research decisions are informed by patient experiences, priorities, and needs
- Ex. PICOTS Framework

Patient Engagement & Patient-Centered Research Methods

Patient Engagement: generally qualitative, examples include:

- Interviews
- Focus groups
- Patient advisory boards
- Listening sessions

Patient-Centered Research: could be qualitative or quantitative; examples include:

- Qualitative research methods
- Surveys or patient preference studies
- Clinical trials
- Observational studies
- Economic evaluations

Source: Adapted from FDA's Patient-Focused Drug Development Guidance Series.

Important factors to move patient-centricity forward

Patient centered means doing things **WITH** patients -- **not FOR or TO** patients

- **Patient & clinical community relationships**
- **Robust patient recruitment network**
- **Plain language & other health literacy principles**
- **Metrics to measure the impact of patient engagement & patient experience data**
- **Case examples & templates**
- **Emerging buy-in from decision-makers**
  - Opportunities to collect or submit patient experience data
  - An expectation that patient experience data are integrated and guide decision-making
  - Transparency about its use

Framework for the Use of Patient Experience Data Throughout the Product Lifecycle

Biotechnology Innovation Organization

Clinical Development

	Pre-IND	EoP1	EoP2	Pre-NDA/BLA	Mid-cycle	Other Type B or C
Current Meeting Opportunities	Pre-IND Meetings Other Type A , B, or C Meetings Critical Path Innovation Meetings INTERACT Meetings (CBER)	EoP1 Meetings Other Type A, B, or C Meetings	EoP2 Meetings Other Type A, B, or C Meetings	Pre-NDA/BLA Meetings Other Type A, B or C Meetings	Mid-cycle Communication Late Cycle Meetings Advisory Committee Meetings	Other Type B or C Meetings
Product Stage	Preclinical Development	Phase I	Phase 2	Phase 3	Health Authority Review and Marketing Authorization	Postmarketing
Examples of Patient Experience Data Applicable to the Product Lifecycle	• Treatment burden • Patient input on protocol designs • Clinical trial burden • Disease burden • Natural history study • Identification of clinical outcome assessments	• Patient preference for treatment • Patient benefit-risk acceptability • Treatment burden • Patient input on protocol designs • Clinical trial burden • Disease burden • Natural history study • Validating clinical outcome assessments • Patient reported outcomes • Quality of life			• Patient risk tolerance • Clinical outcome assessments	• Patient outcome in clinical practice • Clinical outcome assessments • Development of patient support applications
Relevant Decisions made During this Phase of the Product Lifecycle	• Product design (i.e., type of device, how to take the medicine, etc.) • Protocol design (i.e. meaningful endpoints) • Clinical trial participation • Understanding the feasibility of trial participation	• Treatment arm selection • Subpopulation identification • Risk mitigation • Benefit-risk assessment • Clinical outcome Assessment Identification • Clinical trial design • Personalized medicine/biomarker • To inform the development of drug development tools • Eligibility for expedited programs			• Structured benefit-risk assessment • Subpopulation identification • Labeling optimization • Discussion at Advisory Committee meetings • Labeling	• Label/indication expansion • Shared decision making • Personalized medicine/ biomarkers • Quality of care/adherence (i.e., label clarification, physician counseling) • Risk management • Value frameworks

IDEAL OPPORTUNITY TO COLLECT PATIENT EXPERIENCE DATA

Patient Experience Mapping Toolbox

Project Planning

- Introductory video
- Project Coordinator Guide
- Interviewer Training Guide

Data Collection Tools

- Plain language consent form
- Interview Guide Template
- “Map My Experience” visual

Source: Oehrlein EM, Schoch S, Majercak K, Gressler LE, Constantino R, Perfetto EM on behalf of Patient Experience Mapping Working Group. Patient Experience Mapping Toolbox. National Health Council; 2021. Available from: https://nationalhealthcouncil.org/resources/patient-experience-map

Applying Patient Experience Data: Real-World Research

- Understanding the actual start date of medication exposure
- Understanding unmeasured confounder distributions between treatment groups
- Understanding reasons for treatment discontinuation in medication adherence studies
- Identifying endpoints and outcomes relevant to patients and caregivers

Oehrlein EM, Schoch S, Burcu M, et al. **Developing Patient-Centered Real-World Evidence: Emerging Methods Recommendations From a Consensus Process**. *Value Health*. 2023;26(1):28-38. doi:10.1016/j.jval.2022.04.1738

CASE EXAMPLES

Case example: olipudase alfa-rpcp

"Having reached this scientific conclusion, the team then **turned to additional sources for the patient perspective** to inform the overall benefit-risk determination."

Patient-reported outcome measures + clinical measures

"There was **substantial evidence of effectiveness**."

+

Structured interviews with caregivers

Unmet needs: "Parents were clear that there is significant unmet need

Views on risks & safe use: Clear labeling would be sufficient to support doctors and patients in making informed, individualized treatment decisions.

- Niemann-Pick Disease Alliance webinar May 12, 2022

Source: FDA; NDA 215498; Xenpozyme (olipudase alfa-rpcp) Integrated Review

Case example: nedosiran

- The Applicant collected information about patient quality of life and impact on caregivers (Table 4).
- The experiences and perspectives shared by patients and caregivers during an **Externally-led Patient Focused Drug Development meeting** hosted by the Oxalosis and Hyperoxaluria Foundation on October 5, 2020, and the **publications** listed in Table 4 also informed the benefit-risk assessment.

Table 4. Patient Experience Data Submitted or Considered

Check if Submitted	Type of Data	Section Where Discussed, if Applicable
Data Submitted in the Application		
Clinical Outcome Assessment Data Submitted in the Application		
☒	Patient-reported outcome	
☐	Observer-reported outcome	
☐	Clinician-reported outcome	
☐	Performance outcome	
Other Patient Experience Data Submitted in the Application		
☐	Patient-focused drug development meeting summary	
☐	Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel)	
☐	Observational survey studies	
☐	Natural history studies	
☐	Patient preference studies	
☐	Other: (please specify)	
☐	If no patient experience data were submitted by Applicant, indicate here.	
Data Considered in the Assessment (But Not Submitted by Applicant)		
Check if Considered	**Type of Data**	**Section Where Discussed, if Applicable**
☒	Perspectives shared at patient stakeholder meeting	
☒	Patient-focused drug development meeting summary report	
☐	Other stakeholder meeting summary report	
☐	Observational survey studies	
☒	Other: Publications 1. Lawrence and Wattenberg (2020) 2. Milliner et al. (2020)	

Source: Data provided in Application, Externally-led Patient Focused Drug Development meeting hosted Hyperoxaluria Foundation on October 5, 2020, and published literature.

"To identify the concerns of the primary hyperoxaluria community, multiple in-person meetings were convened and a web-based survey was developed by families, the Oxalosis and Hyperoxaluria Foundation (OHF), the Kidney Health Initiative (KHI), and the American Institutes for Research."

Source: FDA; NDA 215842; Rivfloza (nedosiran) Integrated Review

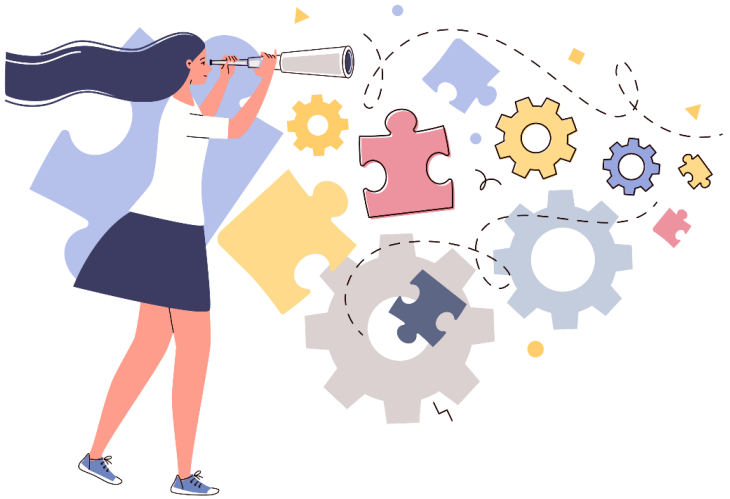
Case Example: Atrial Fibrillation Patient Advisory Board & Implications for RWD Study Designs

EXPERIENCES RECEIVING AN AF DIAGNOSIS

DISCUSSION SUMMARY	TAKEAWAYS	RECOMMENDATIONS
• Patients often present with varying symptoms that go unrecognized as AF by HCPs, causing delayed diagnosis or misdiagnosis • Some patients interpreted their AF symptoms as another condition they had experienced before, which further delayed a correct diagnosis	• Diagnosing AF can be very challenging since it is often transient in nature and symptoms are similar to those of other illnesses/diagnoses • First AF diagnosis code in a database is unlikely to reflect true first onset of AF symptoms	• Consider ways to identify and use codes for prior GI disorders, fatigue, syncope, anxiety, etc. as indicators of first symptom presentation • Sensitivity analyses (using these indicators) may be useful in identifying initial onset of AF among symptomatic patients

Source: Oehrlein EM, Luo X, Savone M, et al. **Engaging Patients in Real-World Evidence: An Atrial Fibrillation Patient Advisory Board Case Example**. *Patient*. 2021;14(2):295-300. doi:10.1007/s40271-020-00479-8

A few opportunities to advance the field today!



ISPOR's Special Interest Groups (SIG)

- Examples: Patient-Centered, Clinical Outcome Assessment, Health Preference Research
- www.ispor.org/member-groups/special-interest-groups

Patient Experience Dossiers

- A template for patient groups to consolidate patient-centered evidence and communicate more efficiently to healthcare stakeholders
- Partnership with National Health Council and Patient-Focused Medicines Development (PFMD)

IVI Patient-Centered Economic Impacts Project

- Upcoming workshops: Explore practical approaches for implementing the framework for patient-centered economic impacts in research framework by engaging patients, caregivers, researchers, and other stakeholders
- Contact: Ushma Patel, IVI's Director of Patient Engagement, ushma.patel@thevalueinitiative.org

Off the Wall: Atlanta's Civil Rights & Social Justice Journey.
180 Williams Street Northwest, Atlanta, GA, 30303