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Improving healthcare decisions

Co-Creating a Clinical Outcome Assessment (COA)-Strategy with Patient Partners: Guidance, Good Practice Methods, and Case Examples

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SECTION

1

Welcome and Introductions

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Moderator & Speakers

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Global PRO Lead
Kyowa Kirin Ltd, UK

ISPOR Task Force Co-Lead

ISPOR Clinical Outcome Assessment SIG and Patient-Centered SIG

- The PC and COA SIGs collaborated on the ISPOR "Patient-Centered Research" Open Meeting following the ISPOR Patient-Centered Research Summit 2024, which inspired the development of this workshop

Agenda

1. Welcome & Introductions
2. Patient Centricity and Engagement in Research – A Short Overview
3. Regulatory Perspective
4. Patient Advocacy Role
5. Industry Perspective

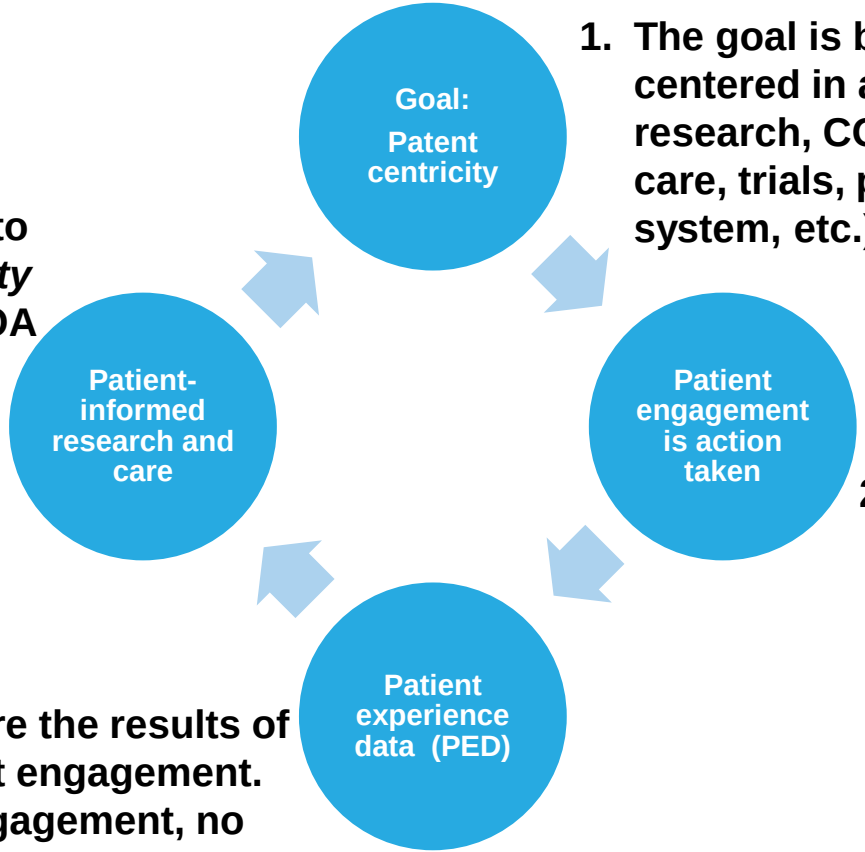
SECTION

2

Patient Centricity and Engagement in Research – A Short Overview

Eleanor Perfetto, PhD, RPh, MS (Moderator)
University of Maryland School of Pharmacy, Baltimore, MD, USA

Some Patient-Centricity Axioms:



1. **The goal is being patient centered in an *activity* (e.g., in research, COA development, care, trials, policy, health system, etc.)**
2. **Patient engagement is the action taken to gather and understand patient experiences.**
3. **PED are the results of patient engagement. No engagement, no PED. (e.g., COAs)**
4. **PED is leveraged to improve the *activity* (e.g., research, COA development)**

Definitions

Term	What it is	What it isn't
Patient* centered	• A focus on patients (& families) and what they say is important to them • Patients playing an active role • Patients engaged as partners • Input patients provide is leveraged to make things better for patients • Doing things WITH patients, not FOR or TO patients	• Saying we put patients "at the center" of all we do • Giving patients whatever they demand • Just including patients in a study as study subjects
Patient* engagement[&]	• Partnership and collaboration among patients and others in research & care • Active, meaningful, real interaction • Recognizing patients' experiences, values, and knowledge • Co-creation • Leveraging patient input to guide and improve engagement	• Placing a single, "token" patient on a committee • Asking patients survey questions to get the answers someone else cares about • Including patients in trials as subjects • Putting some "done" in front of patents and asking for feedback

* The term "*patient*" can include caregivers, family members, and patient groups that represent patients with a disease.

[&] *Engagement* can happen in any part of healthcare such as research or care.

ISPOR Definition of Patient Engagement in Research

- ✓Partnership between patients and researchers
- ✓Active, meaningful, and collaborative interaction
- ✓Across all aspects and stages of the research process
- ✓Research questions and decision-making are guided by patient input
- ✓Patient experiences, values, and knowledge are recognized and valued

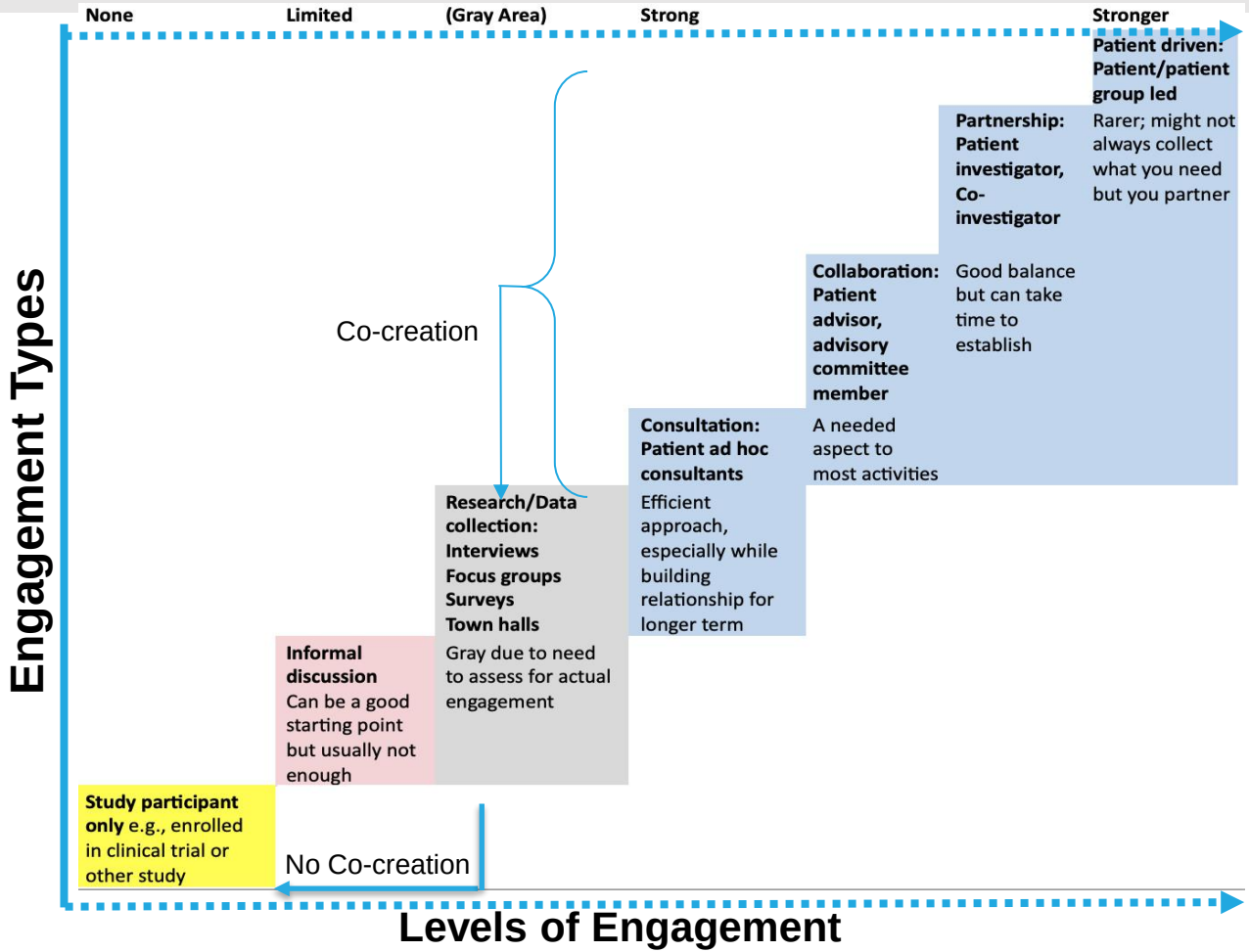
Value in Health. Vol. 22, Iss 6

Patient Experience Data (PED)

FDA's definition:

- Data collected by any persons intended to provide information about patients' experiences with a disease or condition.
- Can be interpreted as information that captures patients' experiences, perspectives, needs, and priorities related to (but not limited to):
    1) symptoms of their condition and its natural history
    2) impact of the conditions on their functioning and quality of life
    3) experience with treatments
    4) input on which outcomes are important to them
    5) patient preferences for outcomes and treatments
    6) relative importance of any issue *as defined by patients*

Defined in Title III, section 3001 of the 21st Century Cures Act, as amended by section 605 of the FDA Reauthorization Act of 2017



Balancing the need for a range of engagement activities

Is it PED Data Collection?

Patent Experience Data Collection

- Interviews
- Focus groups
- Surveys

} Can be a gray area!

Why a possible gray area?

- Interviews, focus groups, and surveys are great methods for collecting PED!
- But, patients need to be engaged in designing the data collection to inform:
  - The questions being asked
  - How questions are asked
  - Words and phrases used
  - Burden, sensitivities, etc.

Just asking patients questions and getting their answers is not enough.
There must be patient engagement in the research design itself.
That is Co-Creation!

Engagement Good Practices: Dimensions and Sample Metrics

1. Patient partnership
2. Transparency
3. Representativeness
4. Diversity
5. Focus is on outcomes patients care about
6. Patient-centered data sources and methods
7. Timeliness

Domain: Patient Partnership

Characteristics of Meaningful Patient Engagement	Examples of Patient Partnership	
	Meaningful ⟷ Insufficient/Low	
Patients are recognized as partners and integrated in all development phases.	A Patient and Family Advisory Council identified a challenge, co-developed a solution with hospital staff, implemented the planned solution, and measured the impact.	A Patient and Family Advisory Council identified a challenge, but hospital administrators and health care providers developed and implemented their solution without input from the Council.

National Health Council Rubric to Capture the Patient Voice

Resources for Engagement and Co-Creation

- Patient-Centered Outcomes Research Institute (US orientation)
  - Engagement in Research Resources
- National Health Council (US)
    - Patient Engagement Rubric
    - Patient Engagement Compensation and Contracting Toolbox (US)
    - Patient Experience Mapping Toolbox
    - Patient-Centered Core Impact Set Toolbox
- Patient-Focused Medicines Development (EU, exUS)
    - Patient Engagement Quality Guidance
    - Fair Engagement Planner (exUS)
    - Global Patient Experience Data Navigator
- EUPATI – Education and Training Courses (EU orientation)

SECTION

3

Regulatory Perspective on the Use of PED for Regulatory Decision-Making

Ashley Slagle, MS, PhD
Aspen Consulting, LLC, Steamboat Springs, CO, USA
Former FDA COA Staff

FDA supports the collection of PED, and encourages its use through the lifecycle of drug development

- FDA values evidence of the lived experience of patients and families
- Critical to thoughtfully implement PED collection strategies with the intention to fill research gaps
  - Have specific objectives in mind that can be achieved with PED – PED is not a box checking exercise!
  - FDA does not value PED for PED sake, but relies on it to make regulatory decisions
- Clinical Outcome Assessments (COAs) as trial endpoints are the most widely used type of PED by the FDA for decision making
  - COA strategy is critically important because evidence from COA endpoints directly contributes to the benefit-risk decisions by FDA
  - Developing the evidence for a COA as fit for purpose requires patient (or family) input

Example: Co-Creating a COA in Early Parkinson's Disease

Morel et al.
Research Involvement and Engagement (2023) 9:98
https://doi.org/10.1186/s40900-023-00505-7

Research Involvement and Engagement

RESEARCH Open Access

The value of co-creating a clinical outcome assessment strategy for clinical trial research: process and lessons learnt

Thomas Morel[1*], Karlin Schroeder[2], Sophie Cleanthous[3], John Andrejack[4], Geraldine Blavat[4], William Brooks[4], Lesley Gosden[5], Carroll Siu[5], Natasha Ratcliffe[5] and Ashley F. Slagle[6]

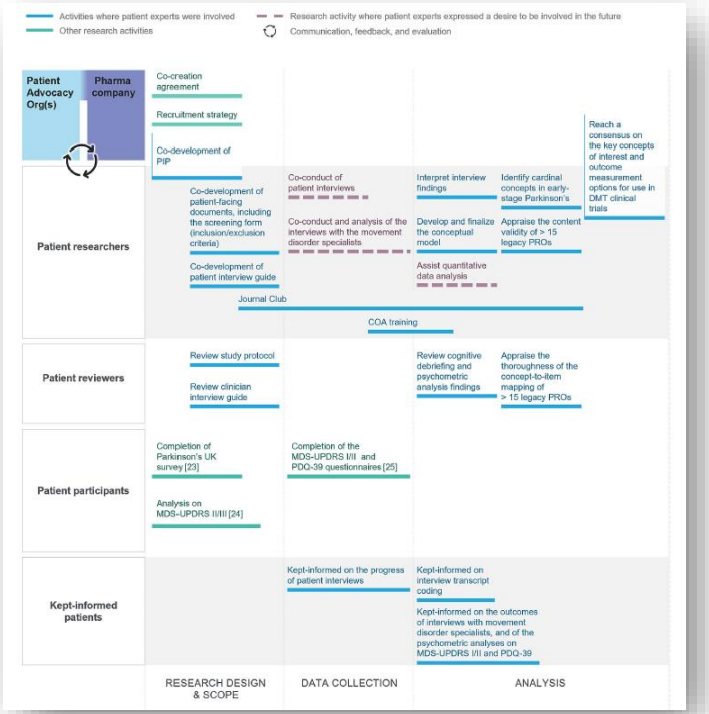
Abstract

Background In support of UCB pharmaceutical research programs, the aim of this research was to implement a novel process for patient involvement in a multidisciplinary research group to co-create a clinical outcome assessment strategy to accurately reflect the experience of people living with early-stage Parkinson's. Patient experts were an integral part of the decision-making process for patient-reported outcome (PRO) research and instrument development.

Methods In partnership with two patient organizations (Parkinson's UK and the Parkinson's Foundation), 6 patient experts were recruited into a multidisciplinary research group alongside clinical, patient engagement and involvement, regulatory science, and outcome measurement experts. The group was involved across two phases of research; the first phase identified what symptoms are cardinal to the experience of living with early-stage Parkinson's and the second phase involved the development of PRO instruments to better assess the symptoms that are important to people living with early-stage Parkinson's. Patient experts were important in performing a variety of roles, in particular, qualitative study protocol design, conceptual model development, and subsequent co-creation of two PRO instruments.

Results Involving people with Parkinson's in PRO research ensured that the expertise of these representatives from the Parkinson's community shaped and drove the research; as such, PRO instruments were being developed with the patient at the forefront. Working with patient experts required considerable resource and time allocation for planning, communication, document development, and organizing meetings; however, their input enriched the development of PRO instruments and was vital in developing PRO instruments that are more meaningful for people with Parkinson's and clinicians.

Conclusions Conducting PRO research, in the context of clinical development involving pharmaceutical companies, requires balancing regulatory and scientific rigor with tight time constraints. Incorporating a multi-stakeholder perspective, which included patient experts as joint investigators, had a strong positive impact on our research, despite the logistical complexities of their involvement. Due to the input of patient experts, the innovative clinical outcome assessment strategy and the co-created novel PRO instruments were more relevant and holistic to the patient experience of early-stage Parkinson's.

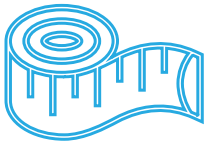


https://researchinvolvement.biomedcentral.com/articles/10.1186/s40900-023-00505-7

Increasing the successful use of PED for regulatory decision-making

- Consider thoughtfully:
  - What are the specific research objectives and what decision(s) will they support
  - How to collect PED
  - How to analyze PED
  - How to communicate PED
    - No single PED dossier for FDA, but incorporate PED appropriately within the entire NDA/BLA submission
- Start planning PED/COA strategy early, generating sufficient evidence for regulatory decision-making takes time
- Regular interactions with the FDA to discuss important PED/COA data that will be the basis for their decision-making
  - No special meeting type for PED, discussions embedded in typical Type B, C, D meetings

While COA labeling is often the goal for sponsors, for approval decisions, FDA considers totality of the evidence, including COA and other PED that may not be labeled



Especially with modest treatment effects, totality of the evidence increases in importance (e.g., exploratory endpoints)

FDA public reviews for NME approvals can be informative

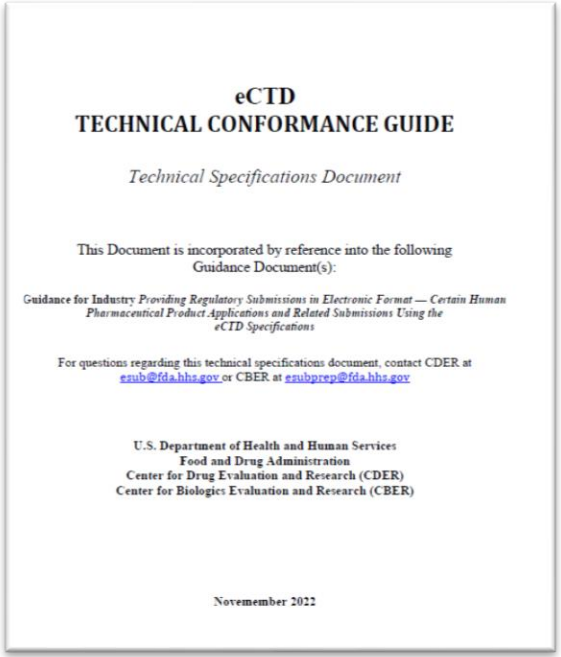
- *https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm*

Electronic Common Technical Document (eCTD) describes how to submit PED to FDA as part of NDA/BLA submissions

3.1.3 Patient Experience Data

If submitting patient experience data as part of an application for marketing approval, the following table should be populated and included in the Reviewer's Guide (section 1.2). Patient experience data (e.g., clinical outcome assessments) collected as part of a clinical trial should be submitted as part of the relevant clinical trial data. Other patient experience data that is separate from clinical trials should be submitted to section 5.3.5.4.

	The patient experience data that was submitted as part of the application, include:	Section(s) and if applicable, file names where data are located and discussed in the application
□	Clinical outcome assessment (COA) data, such as	
□	Patient reported outcome (PRO)	
□	Observer reported outcome (ObsRO)	
□	Clinician reported outcome (ClinRO)	
□	Performance outcome (PerfO)	
□	Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel, etc.)	
□	Patient-focused drug development or other stakeholder meeting summary reports	
□	Observational surveys studies designed to capture patient experience data	
□	Natural history studies	
□	Patient preference studies (e.g., submitted studies or scientific publications)	
□	Other: (Please specify)	



eCTD
TECHNICAL CONFORMANCE GUIDE

Technical Specifications Document

This Document is incorporated by reference into the following Guidance Document(s):

Guidance for Industry *Providing Regulatory Submissions in Electronic Format — Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications*

For questions regarding this technical specifications document, contact CDER at esub@fda.hhs.gov or CBER at esubprep@fda.hhs.gov

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

November 2022

Examples of COA evidence adding to the totality of the evidence and described by FDA in product reviews

trofinetide FDA summary review: *https://www.accessdata.fda.gov/drugsatfda_docs/nda/2023/217026Orig1s000SumR.pdf*

The trial also evaluated the CSBS-DP-IT-SCS in the testing hierarchy (Table 6). Although the results of the CSBS-DP-IT-SCS support the efficacy conclusion, [redacted] (b) (4) [redacted]. Per Dr. Michelle Campbell, associate director for stakeholder engagement and clinical outcomes, there is insufficient evidence to support the use of the scale in this population. Insufficient evidence was provided to justify the administration, scoring, and interpretation of the CSBS-DP-IT-SCS for the population of subjects with Rett syndrome studied. The tool is intended to be a screener in healthy children and was not designed to detect improvement or worsening in communication in the setting of a clinical trial. It is not clear how to interpret the observed difference between treatment and placebo detected by the instrument.

Table 6 Study 003: CSBS-DP-IT-SCS at Week 12

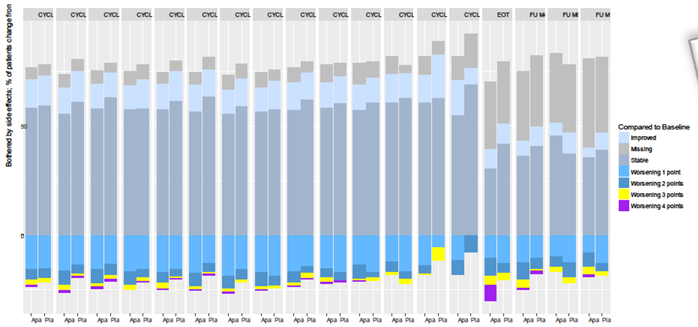
	Placebo (N=93)	Trofinetide (N=91)
Mean baseline value (SD)	8.8 (3.24)	8.7 (0.35)
Week 12 observed mean (SD)	7.5 (2.99)	8.9 (3.74)
MMRM analysis LS mean (SE)	-1.1 (0.25)	-0.1 (0.26)
LS Mean Difference (SE) (trofinetide-placebo)		1.0 (0.37)
95% CI		(0.3, 1.7)
p-value		0.006

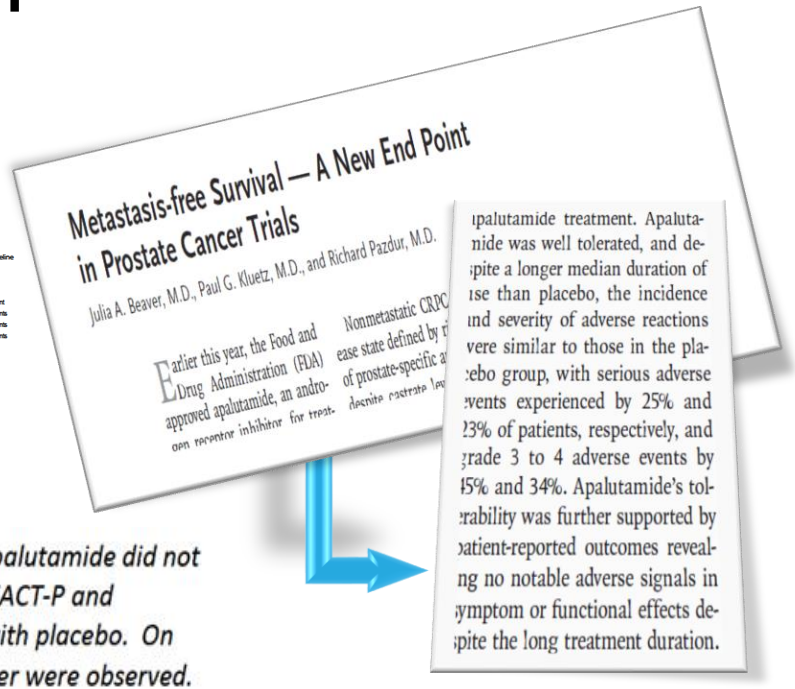
Source: statistical review table 8

Examples of COA evidence adding to the totality of the evidence and described by FDA in product reviews

apalutamide FDA summary review: https://www.accessdata.fda.gov/drugsatfda_docs/nda/2018/210951Orig1s000MultidisciplineR.pdf

Figure 15. Distribution of Change in Response for FACT-P Item GP5 ("I am bothered by side effects of treatment) by Treatment Arm and Cycle





Metastasis-free Survival — A New End Point in Prostate Cancer Trials

Julia A. Beaver, M.D., Paul G. Kluetz, M.D., and Richard Pazdur, M.D.

Earlier this year, the Food and Drug Administration (FDA) approved apalutamide, an andro-

Nonmetastatic CRPC ease state defined by r of prostate-specific a

ıpalutamide treatment. Apaluta- nide was well tolerated, and de- ;pite a longer median duration of ıse than placebo, the incidence ınd severity of adverse reactions vere similar to those in the pla- :ebo group, with serious adverse :vents experienced by 25% and 23% of patients, respectively, and ;rade 3 to 4 adverse events by 15% and 34%. Apalutamide's tol- :rability was further supported by ɔatient-reported outcomes reveal- ng no notable adverse signals in ymptom or functional effects de- ;pite the long treatment duration.

Reviewer's comment: *Exploratory analyses of PROs indicated that apalutamide did not appear to adversely affect functional outcomes as measured by the FACT-P and appeared well-tolerated over a long duration of therapy compared with placebo. On item level review, weight loss and a small increase in side effect bother were observed.*

FDA PED Resources

- Patient Focused Drug Development (PFDD) Guidance Series
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-collecting-comprehensive-and-representative-input
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-methods-identify-what-important-patients
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-selecting-developing-or-modifying-fit-purpose-clinical-outcome
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-incorporating-clinical-outcome-assessments-endpoints-regulatory
- Multiple Endpoints in Clinical Trials Guidance
  - https://www.fda.gov/media/162416/download
- Digital Health Technologies for Remote Data Acquisition in Clinical Investigations Guidance
  - https://www.fda.gov/media/155022/download
- Voice of the Patient Reports
  - https://www.fda.gov/industry/prescription-drug-user-fee-amendments/condition-specific-meeting-reports-and-other-information-related-patients-experience

FDA Virtual Public Workshop: December 13, 2024

PED, including COAs, at FDA and EMA

- FDA outpacing EMA on public guidances and recommendations, patient involvement and methods
- EMA seems a bit more focused on biomarkers and clinician evidence in trials, whereas the FDA is more focused on COAs
- With novel concepts and endpoints, FDA and EMA often discuss
- FDA and EMA are increasingly aligned, though laws and operations are different across the agencies making perfect alignment difficult
  - Both need rigorous PED, including COA, evidence for decision-making

SECTION

4

How to best partner with patients and what "good" looks like

Gunnar Esiason, MBA, MPH (Speaker)
Head of Patient Engagement
RA Ventures, Boston, MA, USA

I've seen the good, the bad and the ugly

BOOMER ESIASON FOUNDATION
BEF
fighting cystic fibrosis

Raven
COMPANY BUILDING WITH RACAPITAL

NPLB No Patient Left Behind

Patient Navigators & Advisory Boards

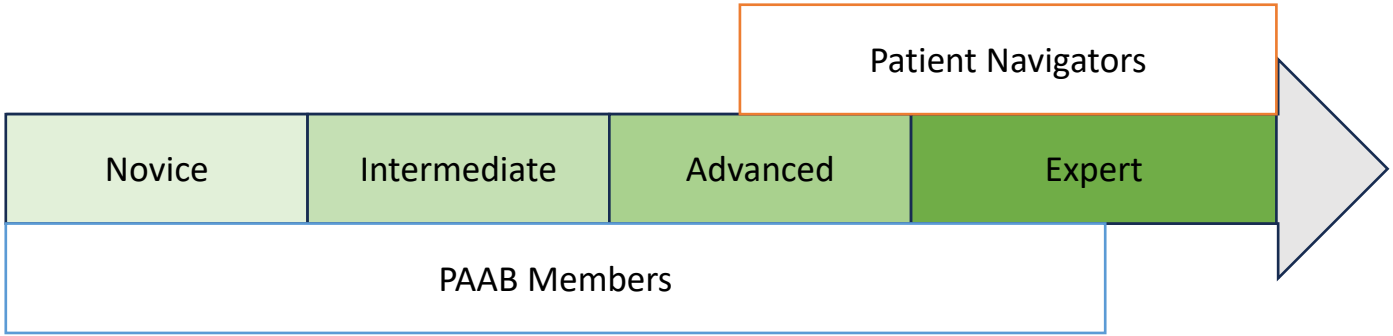
Patient (or caregiver) Navigators

- Patient navigators are contracted patient (or caregiver) advocates who can efficiently guide sponsors through often complex community dynamics, serve as a networker to patient advocacy groups and identify key areas of patient needs.
- They are typically more technically savvy and may have past research or consulting experience with industry.
- Can act quickly and often embed directly into a project team, though may not be precisely representative of specific community.

Patient Advocacy Advisory Boards (PAABs)

- PAABs are more bureaucratic advisory panels that are best built with diverse range of backgrounds and technical life science acumen.
- Important to have a charter in place to govern the board and a project lead associated with the sponsor company who can also convene 1:1 meetings if needed.
- Can be slower to convene and come to consensus on debated items, but often more accurately represents the diverse needs of an individual patient community.

Finding the right partners: patient advocacy groups or individual patient advocates?



Individual Patient Advocates or Partners

- Ability to govern the project as you deem fit
- Requires additional labor to source and seat members per project
- Dependent on the partners sourced to evangelize the output of the project or advisory effort

Patient Advocacy Groups

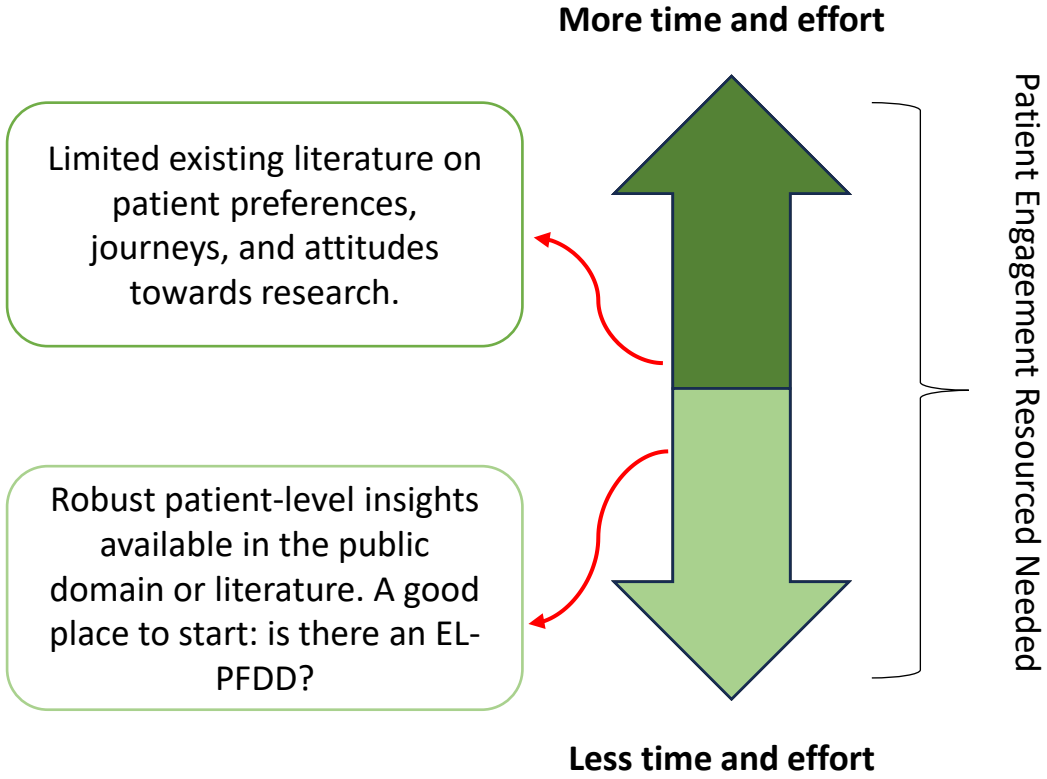
- Ability to leverage existing advisory infrastructure, though typically as a paid service
- Must play by the advocacy groups rules for patient engagement
- If relationship in place, can staff a project quickly

How Much is Too Much, And When is it Not Enough? Resourcing Choices

Patient Engagement is a line item in your budget

- Staffing and employee time
- Consultancy or patient advocacy partnerships
- Compliance timelines
- Background research and access to existing tools

Assess what's out there, and don't reinvent the wheel if you don't have to. Double down when needed, it will pay off in the end.



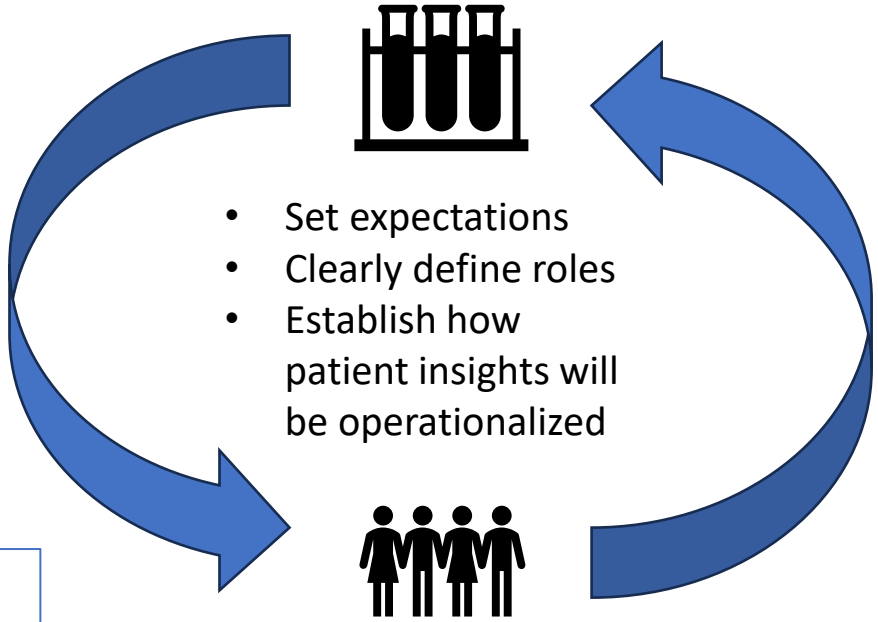
Even in the context of robust output from previous patient engagement exercises, the function should never be overlooked!

Structuring the feedback loop between community members and sponsor companies

At worst, patient engagement can feel patronizing.

Often it can be awkward.

When done well, insights can materially alter a strategy for the better.



Helpful hint for industry: your relationship with your Wall Street analysts isn't awkward, think about your patient partners in the same way

A few things to keep in mind!

You pay your regulatory consultant, your patient advocates are consulting with you, too. Pay them!

- You do not need to overengineer this. Your HR partner should have access to fair market value rates.
- If all else fails, there are resources out there to help.
  - National Health Council (US-based)
  - PEM Suite (Global)

Sourcing patient advocates for your project is as much of a science as it is an art.

- Finding patient groups rich with debate, commentary and opinions of all shapes and sizes exist both on the Internet and adjacent to medical centers or conferences
  - Patient navigators can help
- Sometimes, patient advocacy groups won't have access to the *right* pool of patients advocates for your project

Patient advocates: you can fire your clients

- Partnership is a two-way street. Everyone needs to fulfill that expectation

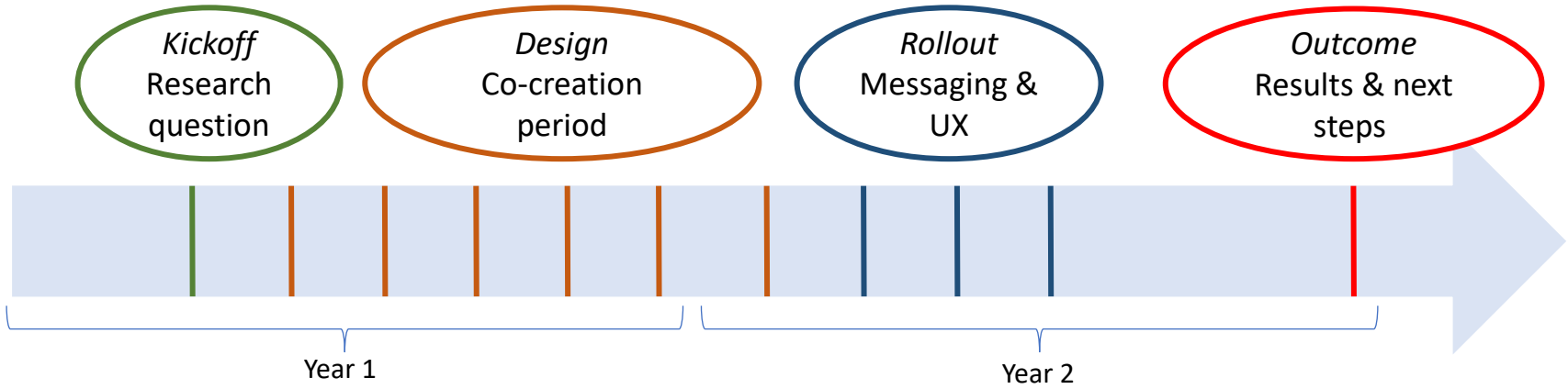
An example of what good looks like

Best Practices

- Clearly establish expectations, process, timeline and cadence
- Lead with topics, guard against *scope creep*, and explicitly call out when patient feedback is used
- Prevent against creating an activity that is overly bureaucratic

Case

- Longitudinal observational study in a rare disease
- Medication adherence and treatment changes following new drug launch



Summary

- Patient advocates, caregiver advocates and advocacy groups are heterogenous in nature and can partner with industry in different ways to achieve a range of goals.
- Sometimes, sourcing the right partner(s) is just as important as the project itself.
- Nothing is free! Plan your resourcing choices thoughtfully.
- Set expectations, align on goals and implement a structured function to absorb patient input into the project team's strategy.

SECTION

5

Industry Case Examples

Angela Rylands
PhD CPsychol
Global PRO Lead, Kyowa Kirin Ltd, UK
ISPOR Task Force Co-Lead

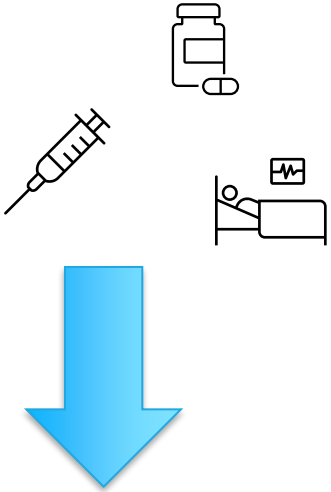
Disclaimer

- My presentation today will cover my personal opinion based on my professional work experience across a number of small and large pharmaceutical and biotech companies
- I will not be giving opinions specific to Kyowa Kirin nor am I giving opinions of other pharmaceutical companies relating to their levels of investment in patient engagement strategies
- I will provide some examples of patient partnership work that I have carried out as part of my role as PRO lead at Kyowa Kirin

Hearing from frustrated patients completing trials led to my own career shift from clinician to industry COA

My Early Career Perspectives:

- Working as a Psychologist on clinical trials
- Long testing periods with patients from multiple therapeutic areas



First-hand feedback from individuals living with different conditions (and their families) told me that the questions asked in the clinical trials we worked on together were *NOT fit for purpose*

Brain scan courtesy of Stock images. Right image courtesy of speaker used with permission from P1vital Ltd. Information courtesy of Angela Rylands.

Industry Aim:
To meet patient needs with
Successful Product

Show V***alue*** of Product

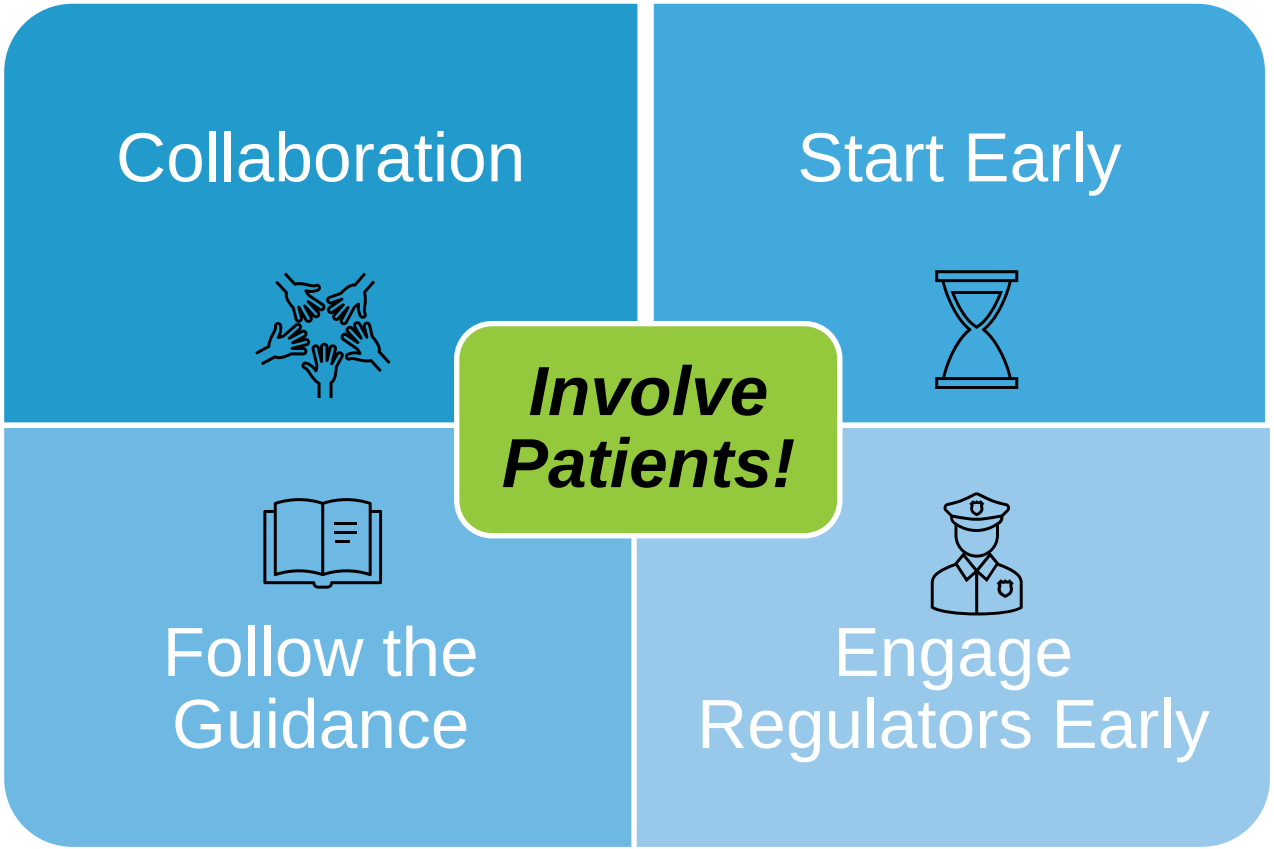
Quantify Value with from Patient Perspective (with Clinical Outcome Assessments, COAs)

Need a ***robust fit-for-purpose COA strategy***

COA Goals!

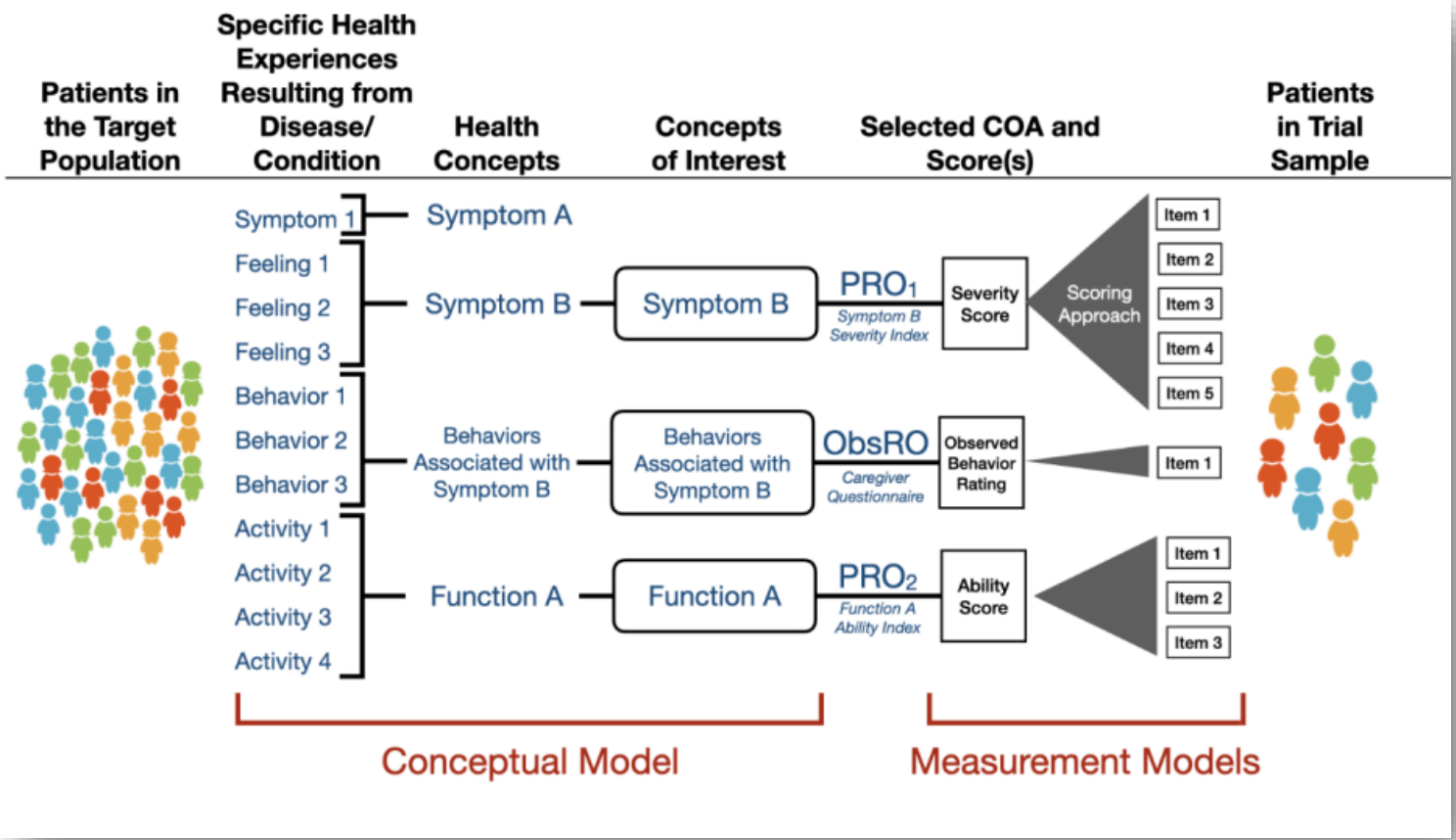
- Image courtesy of Stock images. Information courtesy of Dr Rylands

Industry COA “Key to Success” Toolbox: Ways of Working



Information courtesy of Dr Rylands at Kyowa Kirin

Conceptual Framework forms the foundation of COA Strategy

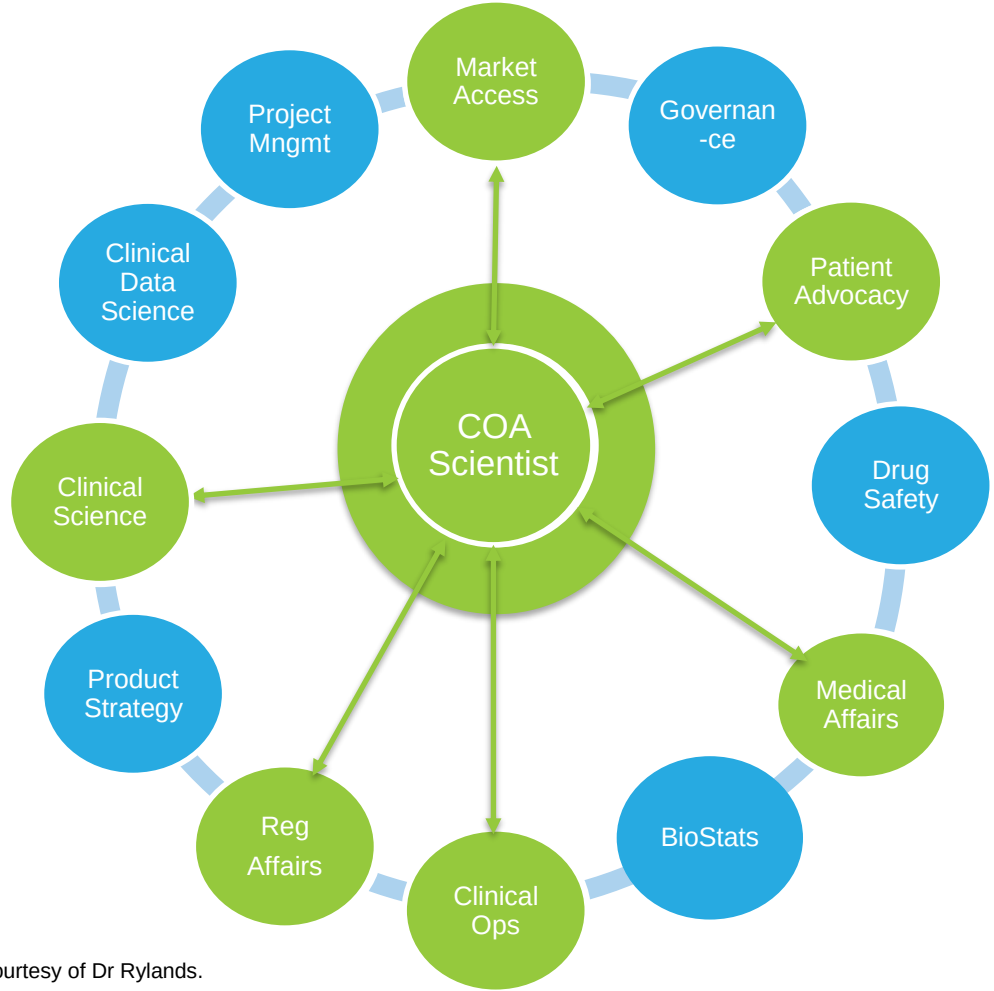


Example (from PFDD) Generic Conceptual Framework Summarizing Which Patient Experiences Will Be Targeted and How They Will Be Measured

Key to success for PP in COA: Internal & External Collaboration

INTERNAL

Cross functional collaboration increases likelihood of having a comprehensive, robust & successful COA Strategy



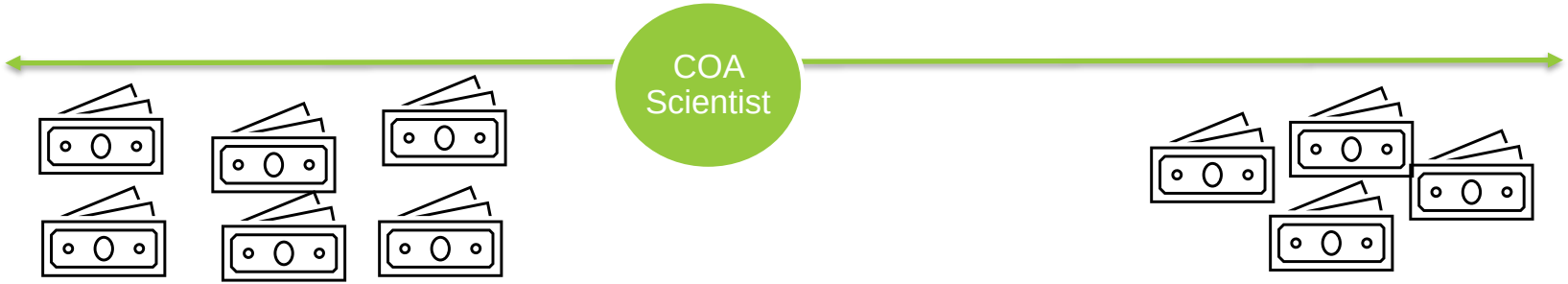
EXTERNAL

Collaboration with Patient & Carer Partners & KOLs for COA related projects

- Information courtesy of Dr Rylands.

Potential Challenges to PP in COA: Infrastructural and Funding

Non-clinical	Phase 1	Phase 2	Phase 3	Post-marketing approval

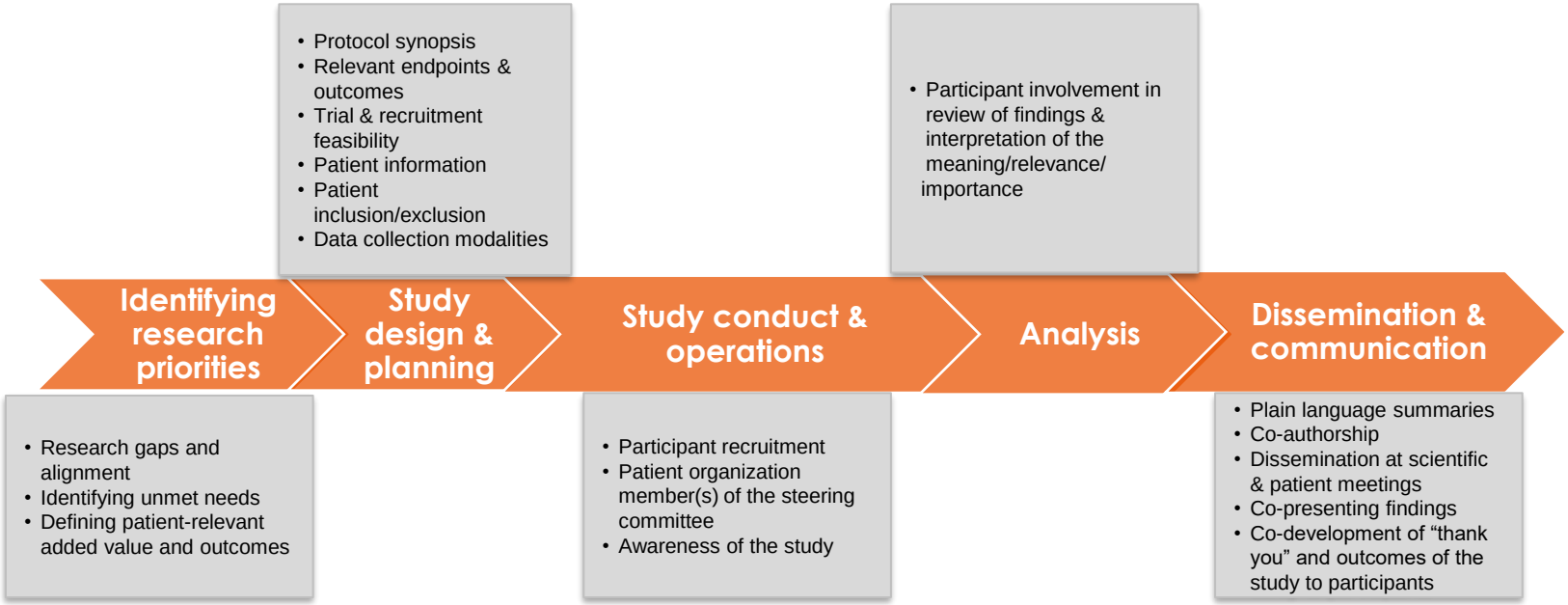


More investment in Patient Partnership work early will reduce the risk that the product will not make it….

Later, more investment in Patient Partnerships is likely e.g. patient support programs, advertising etc.

- Information courtesy of Dr Rylands.

Patient Partners in COA : Throughout Lifecycle of Product



We still have lots more to do to ensure we have patients as partners at every step and we strive to ensure that we are doing this

Figure adapted from Patient involvement roadmap Available at: https://toolbox.eupati.eu/resources-guidance/patient-engagement-roadmap/. Accessed: November 2024.

Early pipeline Example of Patient Partners for COA Strategy

LUPUS ACCELERATING BREAKTHROUGHS CONSORTIUM

Lupus community at front and center of drug development. Sponsors of lupus products, plus clinicians, plus regulators contributing to same goals. Outputs to be used for COA Strategy

Lupus ABC: Public private partnership with FDA in augurated April 2023 to develop initiatives to accelerate lupus drug development

Images and information courtesy of Dr Rylands and Lupus ABC Consortium.

Developing a PRO with Patient Partners

PRO Workshops

Patient centricity in drug development at its best!

Patient and carer partners working with FDA representatives, industry sponsors & actively contributing to workshops

Outputs to be used clinical trials

LUPUS
ACCELERATING BREAKTHROUGHS CONSORTIUM

- Images and information courtesy of Dr Rylands and Lupus ABC Consortium.

Examples Patient Partnerships for COA strategy in Real World Studies

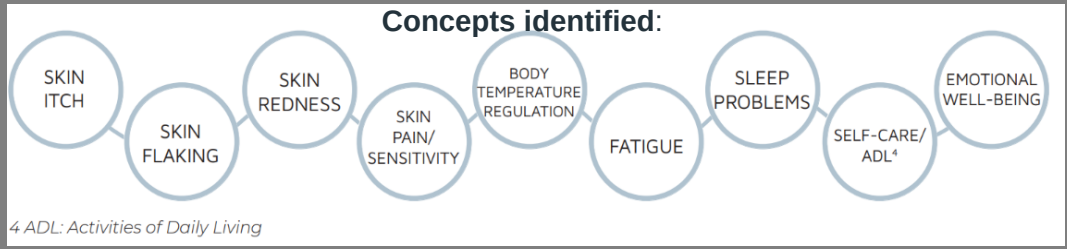
Example 1: Incorporating Patient Experience in Cutaneous Lymphoma[1]

- N=4 Patients & N=3 Spouses Interviewed
- Recruited at hospital clinics

Identified concepts

- Patient experiences of multiple skin-related symptoms (itching, flaking, redness, pain/sensitivity)
- Symptom burden on activities of daily living

Concepts identified:



4 ADL: Activities of Daily Living

Example 2: Adolescent Partners for Rare Bone disease[2]

- N=4 Adolescents & N=1 Carer of 2 Adolescents Telephone Interviews
- Concepts identified
- Input to methodology for wearable and apps over 12 month study

Concepts identified:

Pain, stiffness and tiredness/fatigue had an impact on usual physical activities

Smartphone app

- Daily symptom scores (pain, stiffness, fatigue)
- Diaries for participation in activities
- Time off school/work
- Healthcare resource use
- Health-related quality of life (EQ-5D-Y)

Wearable

- Duration of moderate-vigorous activity
- Step count

Patient interviews

- Symptoms severity
- Symptoms impact on behaviours
- Emotional well-being
- Sleep quality
- Treatment experience
- Future hopes
- Coping strategies

Medical records

- Demographics
- Serum phosphate levels, PTH levels
- Prescribed XLH treatments

Parent interviews

- Understand supportive care needs and burden of carers

[1]Gibson J, et al. Eur J Cancer. 2021;156;pS64, Abstract presented at EORTC 2021 and presented at the **8th Annual European Patients as Partners Conference (London UK 2024),** Saraff et al. A patient-centred and multi-stakeholder co-designed observational prospective study protocol: Example of the adolescent experience of treatment for X-linked hypophosphataemia (XLH). PLoS One. 2024 Jan 19;19(1):e0295080.

[2]Rylands AJ, et al. A patient-centric approach to designing a mixed-methods observational study involving adolescents with XLH. Abstract presented at EU ISPOR, 16–19 November 2020, Virtual: PRO115.

Acknowledgements

Thank you to all our patient partners so far!

XLHuk

Cutaneous Lymphoma Foundation

LUPUS Accelerating Breakthroughs Consortium

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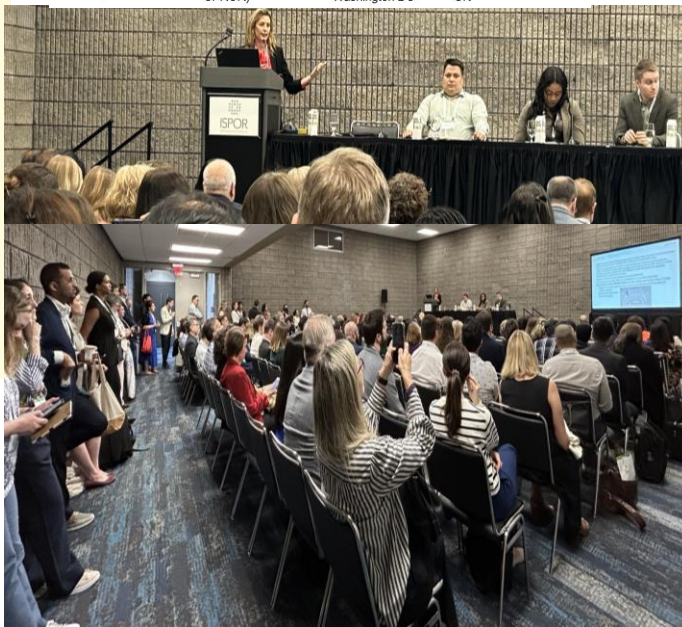
ISPOR Meeting Atlanta, USA 6th May 2024

Angela Rylands (Moderator)
Kyowa Kirin International London, UK

Konrad Maruszczyk (Speaker)
PRO Researcher CPROR)

Onyeka Illoh (Speaker)
FDA COA Division Washington DC

Antony Martin (Speaker)
Health Economist, UK

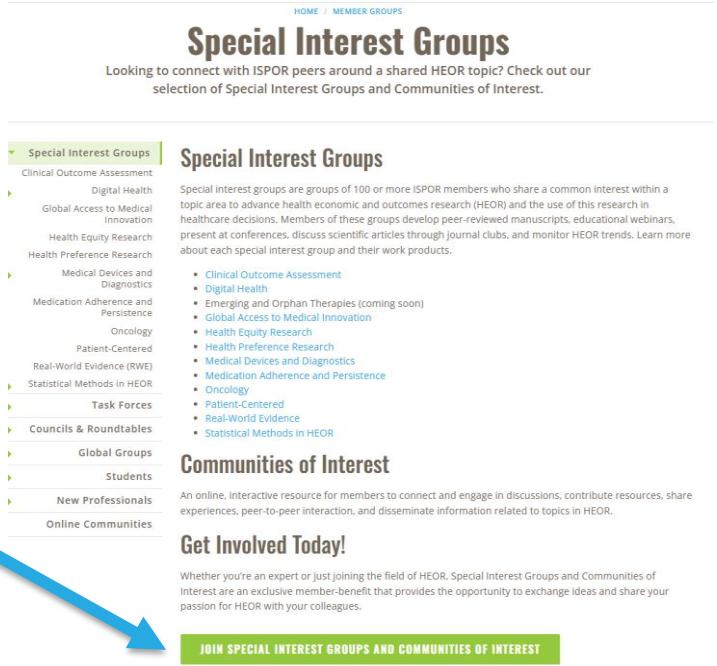


SECTION

6

Audience Participation

Join a Special Interest Group!

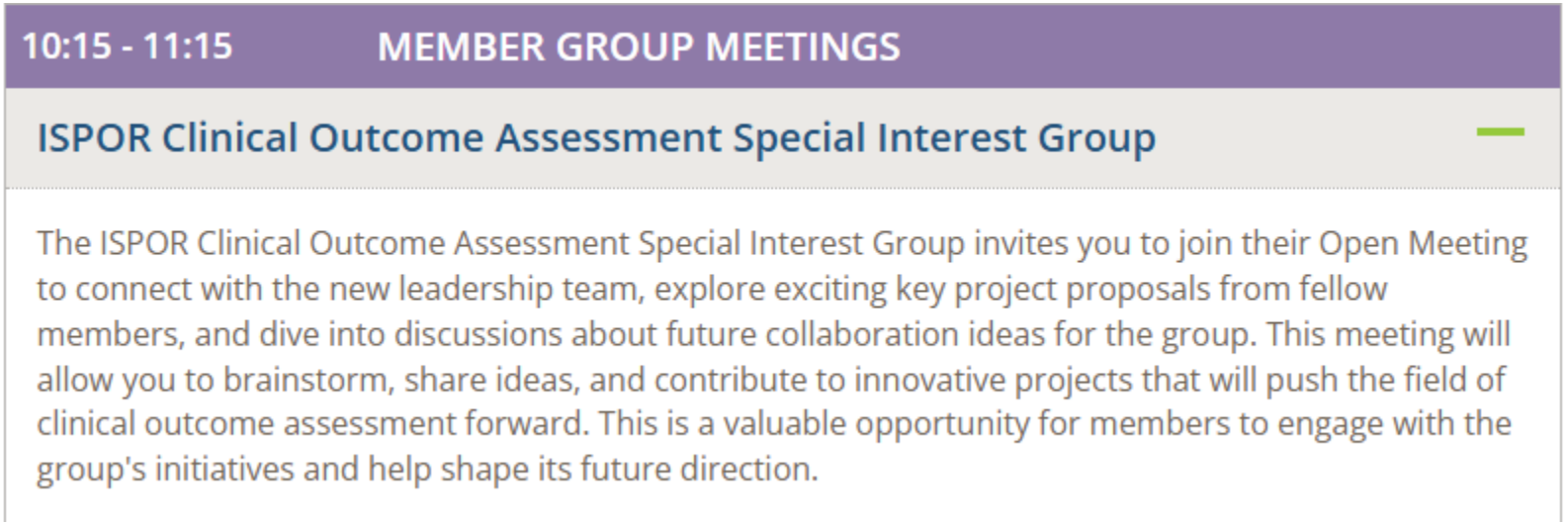


For more information about the Clinical Outcome Assessment SIG or the Patient-Centered SIG, please email ClinicalOutcomeSIG@ISPOR.org or PatientSIG@ISPOR.org.

You must be an ISPOR member to join a Special Interest Group

ISPOR COA SIG Open Meeting – Tomorrow!

- Tomorrow, Tuesday, 19 November from 10:15 – 11: 15 AM
- Room 118-119

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