Making Sense of Sensors

Challenges in Developing Patient-Centered Sensor-Based Outcomes (e.g. Wearables) for Use in Medical Product Decision-Making and Challenges to Adoption in the Real World

Moderator: Katelyn R. Keyloun, PharmD, MS, Director, Data and Digital Strategy, AbbVie

Presenters:

- **Julia K. Garcia,** PhD, MS, Director, PCOR, AbbVie
- **Bill Byrom,** PhD, Vice President and Principal, eCOA Science, Signant Health, UK
- **Andrew Lloyd**, DPhil, Acaster Lloyd Consulting Ltd.

abbvie

SIGNANT HEALTH
THE EVIDENCE GENERATION COMPANY™

AcasterLloyd
Consulting Ltd

Monday, May 6, 2024 | 1:45 PM - 2:45 PM

Intro polling question

Are you leveraging sensor-based outcomes (e.g. wearables) for medical product decision-making?

Select all that apply

a. Yes, as part of Clinical Trials
b. Yes, as part of Regulatory Submissions
c. Yes, as part of Reimbursement/Health Technology Assessments
d. Yes, as part of other types of medical product decision-making
e. Not using, but developing strategy to leverage for future use
f. Not using, but interested

Workshop Overview

Katelyn R. Keyloun (Moderator), PharmD, MS
Director, Data and Digital Strategy, AbbVie

Image source: https://galendata.com/digital-healthcare-future-heathcare/

Rise in Importance of Patient-Centered Outcomes for Regulatory and Reimbursement/HTA Decision-making

Recent Drivers Supporting the Need for Patient-Centered Outcomes in Healthcare Decision-Making

Pricing Negotiation Changes

Upcoming Joint Assessments

European Commission

Joint Council Assessment Framework

Rise in Patient-Centered Initiatives


Therapeutic Innovation & Regulatory Science (2024) 58:63–78
https://doi.org/10.1007/s43441-023-00573-7

DIA

ORIGINAL RESEARCH

Patient Engagement and Patient Experience Data in Regulatory Review and Health Technology Assessment: A Global Landscape Review

Neil Bertelsen[1] · Lode Dewulf[2] · Silvia Ferrè[3] · Rebecca Vermeulen[4] · Karlin Schroeder[5] · Laureline Gatellier[6,7] · Ify Sargeant[8] · Daniela Luzuriaga[9] · Hayley Chapman[9] · Nicholas Brooke[9]

Received: 3 April 2023 / Accepted: 9 August 2023 / Published online: 24 September 2023
© The Author(s) 2023

Value for Regulatory decision-makers

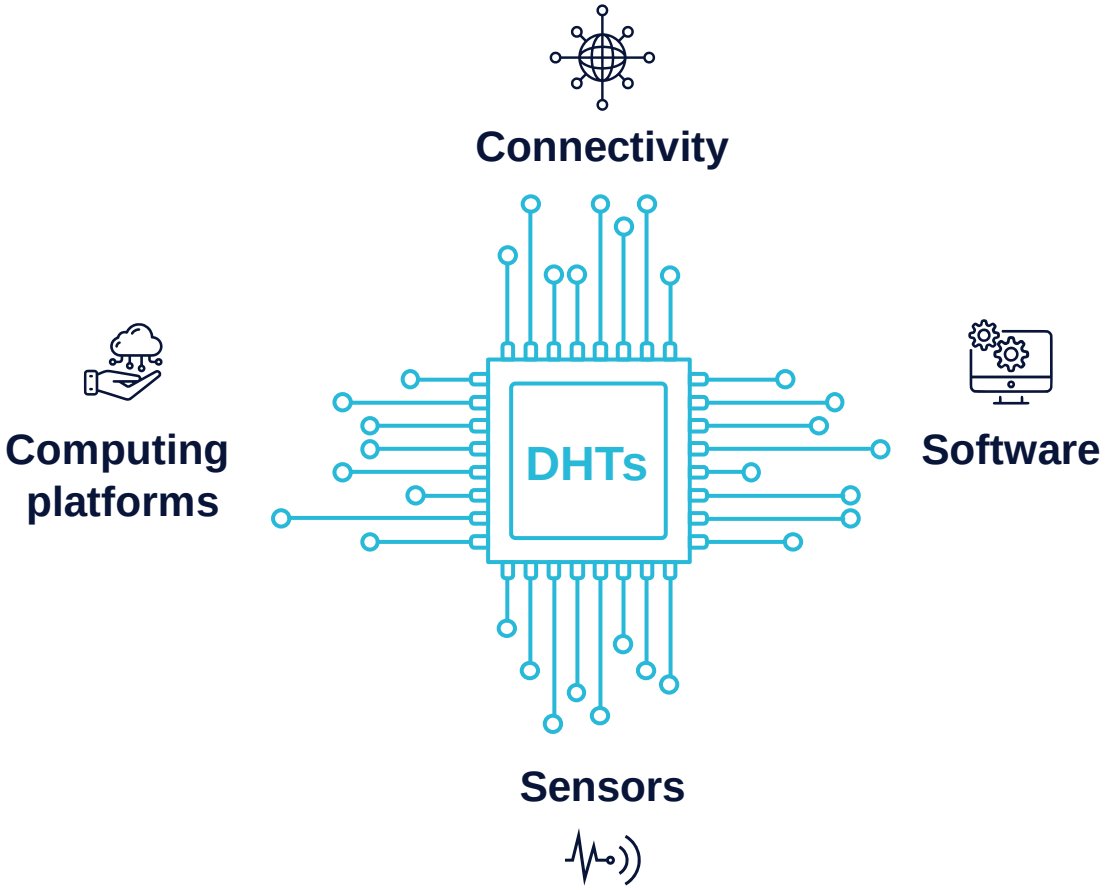
- Support benefit/risk decisions
- Determine meaningful improvement
- Support new indications/label expansions

Reimbursement/HTA decision-makers

- Support value-based decisions to manage risk and cost
- Support evidence on real-world effectiveness

HTA – Health Technology Assessment; References: Bertelsen, N., Dewulf, L., Ferrè, S. et al. Patient Engagement and Patient Experience Data in Regulatory Review and Health Technology Assessment: A Global Landscape Review. Ther Innov Regul Sci 58, 63–78 (2024). https://doi.org/10.1007/s43441-023-00573-7

What is Sensor-Based Digital Health Technology (DHT) and How Can It Be Used to Measure Patient-Centered Outcomes?



DHT are “systems that use computing platforms, connectivity, software, and/or sensors for healthcare and related uses”

Sensor-Based DHT may be external, ingestible, or implantable and can exist on, outside, or within a patient to measure health status

Clinical Outcomes measure how a patient feels, functions or survives (e.g. Patient-reported Outcomes, PROs)

References: https://www.fda.gov/medical-devices/digital-health-center-excellence/what-digital-health, accessed 3/25/24; Sensor-Based DHT definition adapted from: Walton MK, Cappelleri JC, Byrom B, et al. Considerations for development of an evidence dossier to support the use of mobile sensor technology for clinical outcome assessments in clinical trials. Contemp Clin Trials. 2020 Apr;91:105962.

Increasing Regulatory & Industry Recommendations on Leveraging Sensor-Based DHT to Develop Clinical Outcomes ≠ Labeled Endpoints

Regulatory Policy and Activity

2018

Wearable data evidentiary requirements for regulatory decision-making
(Byrom et al. June 2018)

2020

Qualification of digital technology-based methodologies
(June 2020)

Evidence dossier to support the use of mobile sensor technology for COAs in clinical trials
(Walton et al. Apr 2020)

2022

PFDD Guidances
(June 2022*)

Steps for selecting & developing novel endpoints from DHTs
(Apr 2022)

Patient technology implementation framework
(Sep 2023)

DHTs in remote data acquisition for clinical trials
(Dec 2023)

Industry guide for developing & deploying digital clinical measures & remote monitoring
(Oct 2023)

2024

Call to action for harmonized terminology
(Leyens et al. Jan 2024)

Industry Consortia Activity

Limited examples of Sensor-Based DHT used to support Clinical Outcomes in labelling

PFDD: Patient-Focused Drug Development; Source: Modified from Signant Health presentation; DHT: digital health technology; COA: clinical outcome assessment; *Guidances 3 and 4 specifically discuss consi
for DHTs

Increase in Interest from Payers in Leveraging Sensor-Based DHT Clinical Outcome Data ≠ Informing Decisions on Risk and Cost

Payer Initiatives

2015

UnitedHealthcare | UnitedHealthcare Motion®

Consumer-grade fitness trackers for US-based wellness programs **(2015)**

2018

HAS

Guide on assessment of connected medical devices **(January 2019)**

HTx Next Generation Health Technology Assessment

EU funded initiative to create a framework for Next Generation HTA **(January 2019)**

2020

NICE National Institute for Health and Care Excellence

Evidence standards framework for DHTs **(March 2019)**

aetna® Attain by Aetna℠ app

Consumer-grade fitness trackers for US-based wellness programs **(2019)**

efpia ISPOR Improving healthcare decisions

Digital Endpoints Ecosystem & Protocols initiative founded to improve efficiency in developing novel clinical endpoints **(November 2020)**

HEALTH BY SWEDEN

Swedish Vision 2025 for eHealth supports harnessing opportunities offered by digitisation & eHealth **(2020)**

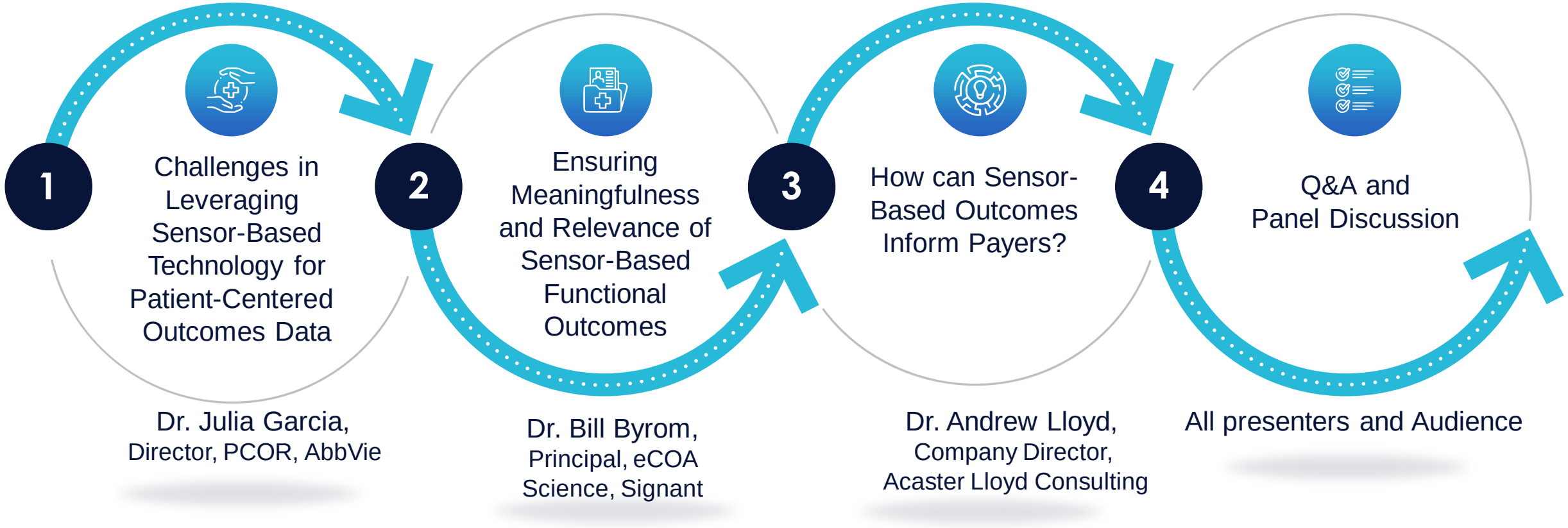
Limited examples of Sensor-Based DHT to inform reimbursement/HTA decision-making

https://www.nice.org.uk/about/what-we-do/our-programmes/evidence-standards-framework-for-digital-health-technologies; https://www.has-sante.fr/upload/docs/application/pdf/2020-05/rapport_dmc_2019_english_vd.pdf
https://www.ispor.org/publications/journals/value-outcomes-spotlight/vos-archives/issue/view/november-2020-supplement-spotlight-on-europe/the-digital-endpoints-ecosystem-and-protocols-(deep)-initiative-a-collaborative-multi-stakeholder-approach-to-defining-and-developing-standards-for-digital-endpoints; https://ehalsa2025.se/wp-content/uploads/2021/02/Strategy-2020-2022_eng.pdf

Workshop Overview

Learning Objectives

1. Identify challenges and best practices in leveraging sensor-based DHT for patient-centered outcomes
2. Explore strategies to improve adoption in payer decision-making



Please have your phones and polling app ready!
We have questions for the audience during Session 1 and Q&A

Challenges in Leveraging Sensor-Based Technology for Patient-Centered Outcomes Data

Julia K. Garcia, PhD, MS
Director, PCOR, AbbVie

A Comprehensive Understanding of Patients' Lived Experience through Sensor-Based DHT for Regulatory & Payer Decision Making

Patient voice is vital for successful medical product development

COAs & Sensor-based DHT enable collection of patient experience data*

An integrated measurement strategy focused on patient-centered outcomes allows us to holistically evaluate the patient experience

Patient experience data informs risk-benefit decision making for regulators and value assessment for reimbursement & access

EXAMPLE: Integrated Measurement Approach to Understand Patient Functioning

Condition	Sensor-Based Clinical Outcomes	COAs
Parkinson's Disease	Motor functioning; e.g., level of tremor during phone calls	Clinician- and patient-reported impact of motor symptoms on quality of life
Atopic Dermatitis	Number of scratching episodes; nocturnal scratching episodes	Patient-reported itch severity; sleep quality
Rheumatoid Arthritis	Activity in the first three hours after awakening	Clinician- and patient-reported number of swollen and tender joints

DHT: digital health technology; COA: Clinical Outcome Assessment

*Patient experience data: data about a patient's experience with a disease or condition

Challenge #1: What is the Appropriate Term for Sensor-Based Technology used to Generate Patient-Centered Outcomes Data?

Background

This is a multidisciplinary field

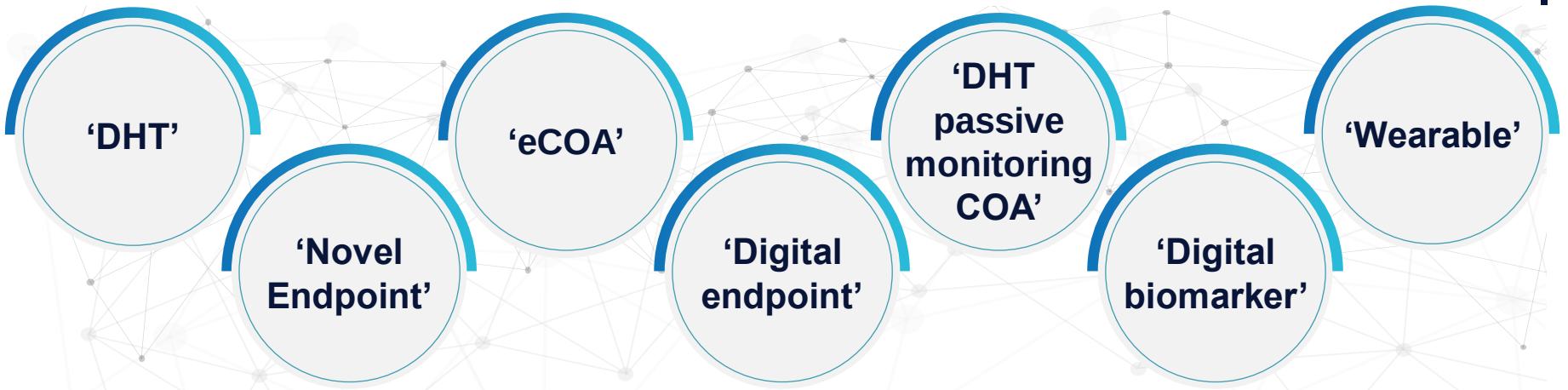
- Measurement science
- Digital health technology
- Analytics
- Patient engagement

Challenge #1

Lack of consensus on appropriate terminology

Is it a type of COA?	If so, what type? PerfO, ObsRO, ClinRO, PRO, new 5th?
Is it a mode of administration like eCOA?	Is it similar to a biomarker?

Are we speaking the same language?



Need aligned terminology to direct us to the type of evidence required

ClinRo: clinician-reported outcome; COA: clinical outcome assessment; eCOA: electronic outcome; DHT: digital health technology; ObsRo: observer-reported outcome; PerfO: performance outcome; PRO: patient-reported outcome

For Consideration: "Sensor-Based Functional Outcomes (SBFOs)"

Sensor-Based Functional Outcomes (SBFOs)

Non-task-based functional outcomes collected and derived leveraging mobile sensor technology directly from patients in both clinical and real-world settings

Includes the term:

- "sensor" (type of DHT data)
- Focuses on functional aspects of clinical benefit (as opposed to feeling or survival)
  Because it is assessing functioning, it is feasible that it is a type of COA with the right level of evidence

Clinical Benefit:
A positive clinically meaningful effect on how an individual feels, functions, or survives*

An SBFO may relate directly or more indirectly to clinical benefit but regardless of where it falls on the continuum it measures a meaningful aspect of health to the patient

[a] Intended to assess how a person feels or functions. [b] Surrogate endpoints and intermediate endpoints.
Source: Manuscript under review; Expert Review of Pharmacoeconomics & Outcomes Research; Multidisciplinary expert team convened with COA/eCOA development expertise, Digital Health expertise, and Regulatory expertise from AbbVie and external authors
*Definition from BEST (Biomarkers, EndpointS, and other Tools) Resource

Polling question about terminology

What is your opinion on the proposed terminology—Sensor-based Functional Outcome (Non-task-based functional outcomes collected and derived leveraging mobile sensor technology directly from patients in both clinical and real-world settings)?

Select all that apply

a. It is clear
b. It is useful
c. I need more time to better understand it
d. It may not accurately convey the intended meaning
e. It may not provide the best solution

Challenge #2: How Can We Ensure the Outcome is Relevant & Meaningful to Patients?

Background

Evidentiary Requirements for COA Development

- Identify concepts meaningful to measure
- Identify/develop outcome measures that map to the concept of interest & context of use
- Confirm outcomes capture meaningful aspects of health from the patient perspective
- Establish meaningful change thresholds

Challenge #2

- *How do COA development methods apply to SBFOs?*
- *Can we use FDA's Patient-Focused Drug Development (PFDD) guidance for COA development?*

COA: clinical outcome assessment; DHT: digital health technology; SBFOs: sensor-based functional outcomes

Challenge #2: How Can We Ensure the Outcome is Relevant & Meaningful to Patients?

Illustrative Example- Congestive Heart Failure

Concept Identification via Patient Interviews	
Walking grandchildren from school	
Do more for myself around the house	

Outcomes Assessed via Sensors	
Time spent sitting/lying down	Sit-to-stand transitions
Number of walking episodes	Time spent in walking episodes

Patients may not describe concepts associated with their condition in terms of a concept measured by an SBFO

SBFOs: sensor-based functional outcomes

Challenge #3: What Role Can an SBFO Play in Informing Payer Decision-Making?

Background

Example:

Congestive Heart Failure

Outcomes Relevant to Payers

Health State Utility	
• EuroQol 5 Dimension (EQ-5D) • Minnesota Living with Health Failure (MLHF) Questionnaire	• Hospitalizations • Emergency room visits

Challenge #3

It is unknown what evidence is necessary to ensure data derived from SBFOs can be used to support payers

- *What role can an SBFO play along with other COAs in informing payer decision-making?*
- *Is there value to payers?*

SBFOs are not currently used to assess health state utility, and the concepts measured with them may be abstract

SBFOs: sensor-based functional outcomes

Conclusions

Challenge #1:

- Lack of appropriate terminology
- Possible solution “Sensor-based Functional Outcomes (SBFOs)”

Challenge #2:

How do COA development methods apply to SBFOs?

Challenge #3:

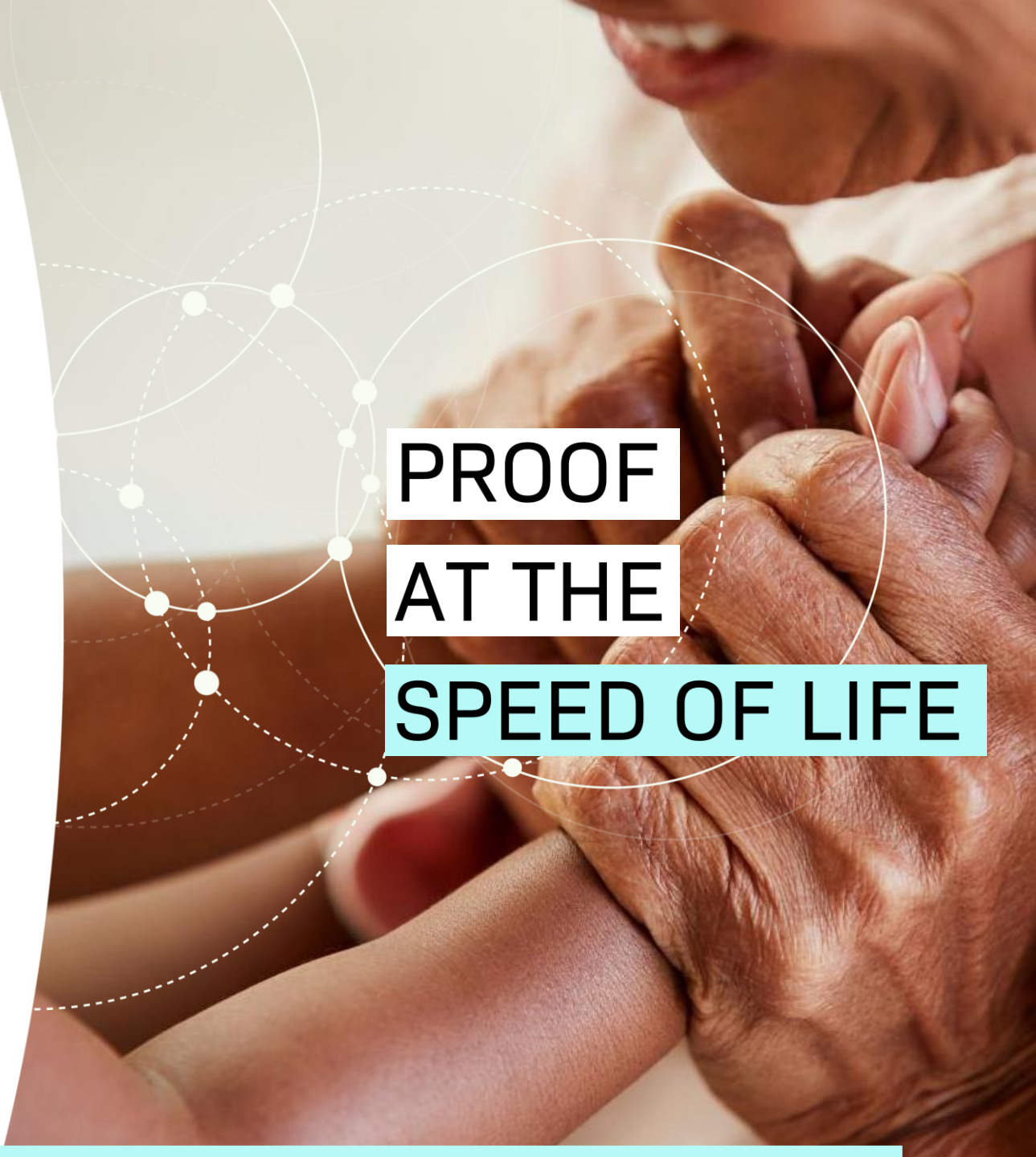
What role can an SBFO play along with other COAs in informing payer decision making?

COA: clinical outcome assessment; SBFOs: sensor-based functional outcomes

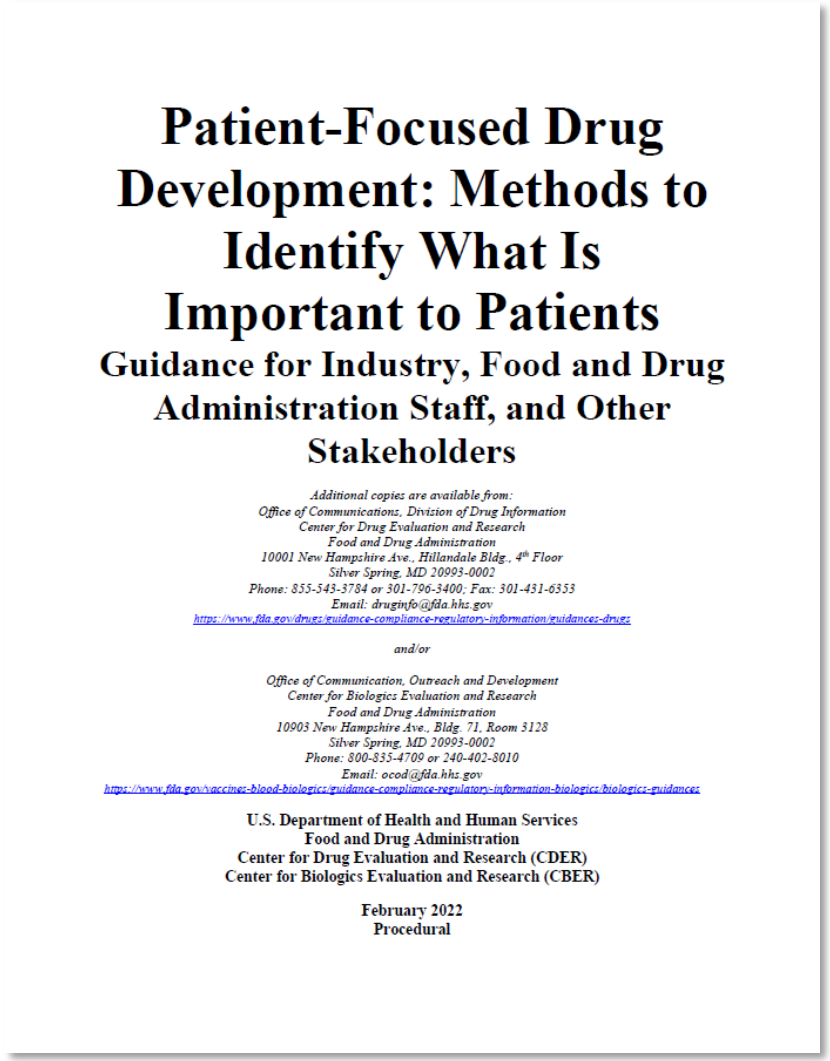
SIGNANT HEALTH
THE EVIDENCE GENERATION COMPANY™

Ensuring meaningfulness and relevance of SBFOs

Bill Byrom, PhD
Principal, eCOA Science, Signant Health



Challenges with the well-trodden approaches



Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

Additional copies are available from:
Office of Communications, Division of Drug Information
Center for Drug Evaluation and Research
Food and Drug Administration
10001 New Hampshire Ave., Hillandale Bldg., 4th Floor
Silver Spring, MD 20993-0002
Phone: 855-543-3784 or 301-796-3400; Fax: 301-431-6353
Email: druginfo@fda.hhs.gov
https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs

and/or

Office of Communication, Outreach and Development
Center for Biologics Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., Bldg. 71, Room 3128
Silver Spring, MD 20993-0002
Phone: 800-835-4709 or 240-402-8010
Email: ocod@fda.hhs.gov
https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances

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)

February 2022
Procedural

ePRO CONSORTIUM
CRITICAL PATH INSTITUTE

Chronic Heart Failure Working Group
SBFO: physical activity

Challenges

- Participant narratives focus on physical activities related to **accomplishing tasks or relating to others**, less so on individual activity components captured by activity monitor devices

  Example: “Playing with grandchildren”

- As the majority of meaningful activities mentioned by participants were **complex** and **task-oriented**, there were notable challenges in discussing the physical activity dimensions measured by an activity monitor

  Example: Step counts or sit-stand transitions

Source: Milena Anatchkova, PhD, Evidera, Inc. PRO Consortium Workshop, 2021

There’s more than one way to skin a cat

Developing meaningful outcome measures

Con•cept el•i•cit•a•tion

The process by which concepts (e.g., symptoms / impacts) that are important to patients are identified through patient interview

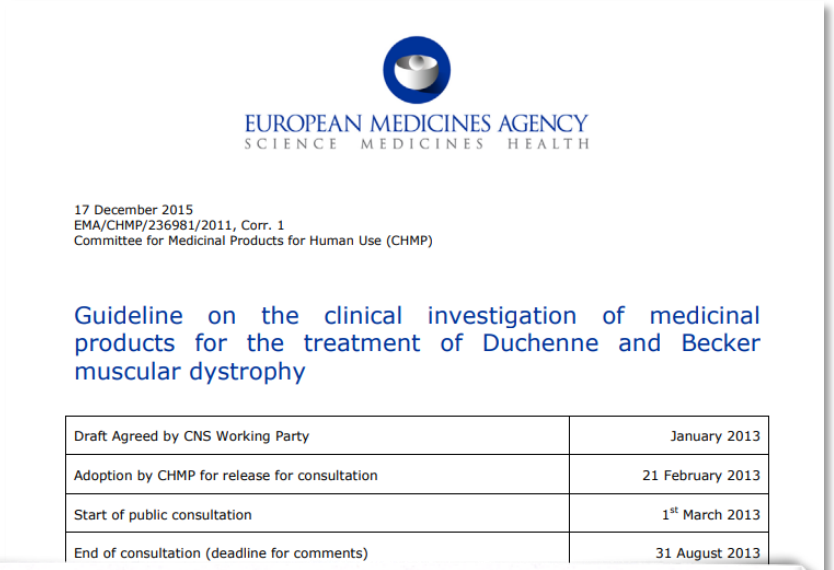
Prospective evidence-led approach

- Traditional concept elicitation

Retrospective evidence determination

- Scientific consensus-driven measures
- Statistically-derived measures
- Retrospective mapping

Scientific consensus-driven approaches



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

17 December 2015
EMA/CHMP/236981/2011, Corr. 1
Committee for Medicinal Products for Human Use (CHMP)

Guideline on the clinical investigation of medicinal products for the treatment of Duchenne and Becker muscular dystrophy

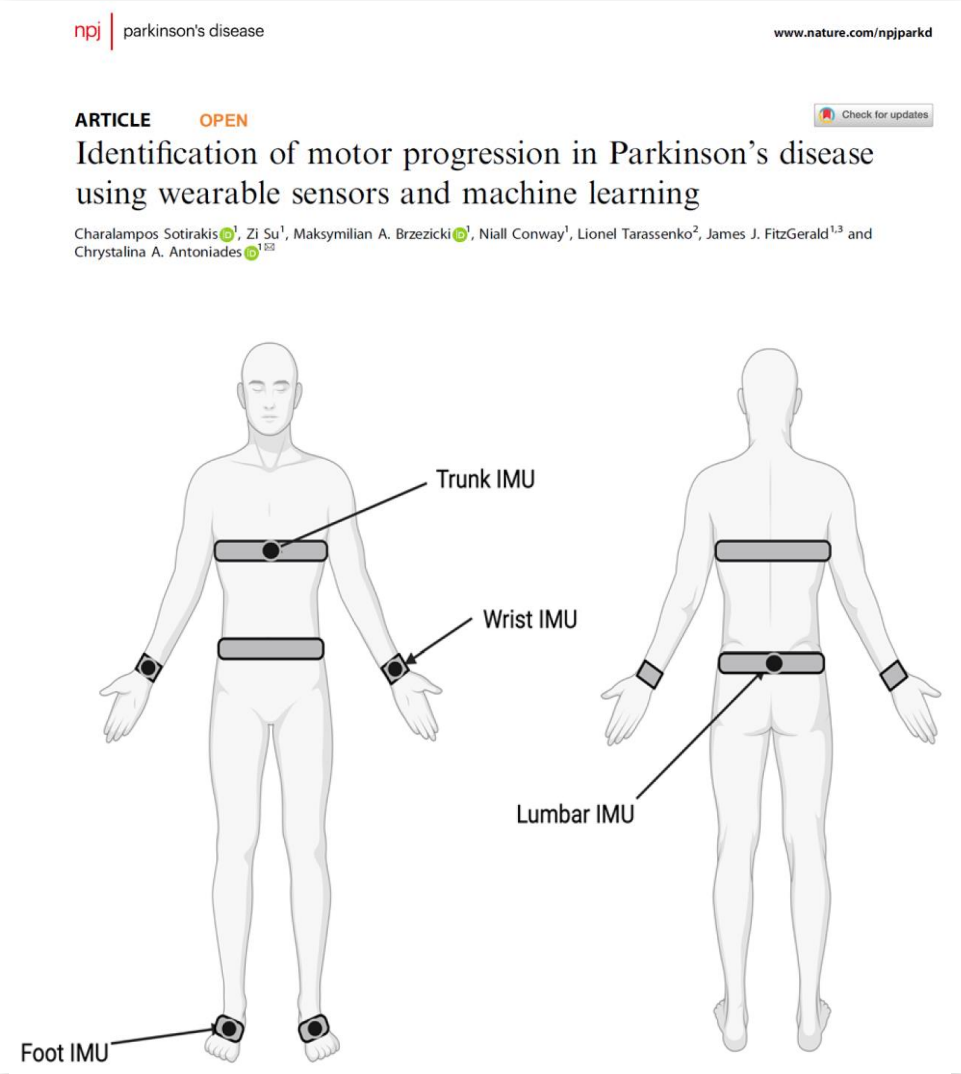
Draft Agreed by CNS Working Party	January 2013
Adoption by CHMP for release for consultation	21 February 2013
Start of public consultation	1st March 2013
End of consultation (deadline for comments)	31 August 2013

> Two endpoints should be selected from the domains **muscle strength** (depending on the functional status and the compound tested) and **motor function**.
>
> Assess muscle strength and motor function **independently** with two endpoints

Sysnav DMD SBFO development

- DMD is affected by progressive muscle weakness, muscle fatigue and joint contractures
- Natural gait velocity is reduced due to decreased step length and reduced cadence
- 6MWT prone to learning effects, episodic variability, AND motivation – can be insensitive endpoint
- At home, real-life measures proposed to be more realistic
  - Distance walked / step count can vary a lot (e.g., weather conditions), gait parameters considered more robust
  - Used gait experts to propose a set of potential gait measures for consideration:
    - Stride velocity: 95th percentile, median
    - Stride length: 95th percentile, median
    - Distance walked/recorded hour

Statistically-derived measures



npj | parkinson's disease www.nature.com/npjparkd

ARTICLE OPEN

Check for updates

Identification of motor progression in Parkinson's disease using wearable sensors and machine learning

Charalampos Sotirakis[1], Zi Su[1], Maksymilian A. Brzezicki[1], Niall Conway[1], Lionel Tarassenko[2], James J. FitzGerald[1,3] and Chrystalina A. Antoniades[1]✉

> *Wearable sensors and machine learning can track the motor symptom progression in people with PD better than the conventionally used clinical rating scales*

N = 91 (74 completers)

	BL	3 m	6 m	9 m	12 m	15 m	18 m
UPDSR-III (motor)	X	X	X	X	X	X	X
2MWT 15-meter length, with U-turns	X	X	X	X	X	X	X
Postural Stability test Standing still, eyes closed, 30 seconds	X	X	X	X	X	X	X

- Body-worn sensors generated **122 parameters** measuring aspects of **gait**, **turning**, and **sway**
- Reduced to a set of **29 parameters** found to change over the time interval
- Random Forest regressor model showed **significant progression of PD symptoms which was not detected by UPRDS-III**

Retrospective mapping: Watch PD Example

Journal of Parkinson's Disease 13 (2023) 589–607
DOI 10.3233/JPD-225122
IOS Press

Research Report

Mapping Relevance of Digital Measures to Meaningful Symptoms and Impacts in Early Parkinson's Disease

Jennifer R. Mammen[a,*], Rebecca M. Speck[b], Glenn T. Stebbins[c], Martijn L.T.M. Müller[b], Phillip T. Yang[d], Michelle Campbell[e], Josh Cosman[f], John E. Crawford[1], Tien Dam[g], Johan Hellsten[h,1], Stella Jensen-Roberts[d], Melissa Kostrzebski[d,i], Tanya Simuni[j], Kimberly Ward Barowicz[b], Jesse M. Cedarbaum[k,l], E. Ray Dorsey[d,i], Diane Stephenson[b] and Jamie L. Adams[d,i]
[a]*University of Rhode Island, College of Nursing, Providence, RI, USA*
[b]*Critical Path Institute, Tucson, AZ, USA*
[c]*Department of Neurological Sciences, Rush University Medical Center, Chicago, IL, USA*
[d]*Center for Health + Technology, University of Rochester Medical Center, Rochester, NY, USA*
[e]*Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA), Silver Spring, MD, USA*
[f]*Abbvie Inc., North Chicago, IL, USA*
[g]*Biogen, Cambridge, MA, USA*
[h]*H. Lundbeck A/S, Valby, Denmark*

It's very similar to typing.

…this one was the Parkinson's symptom that I'm noticing the most.

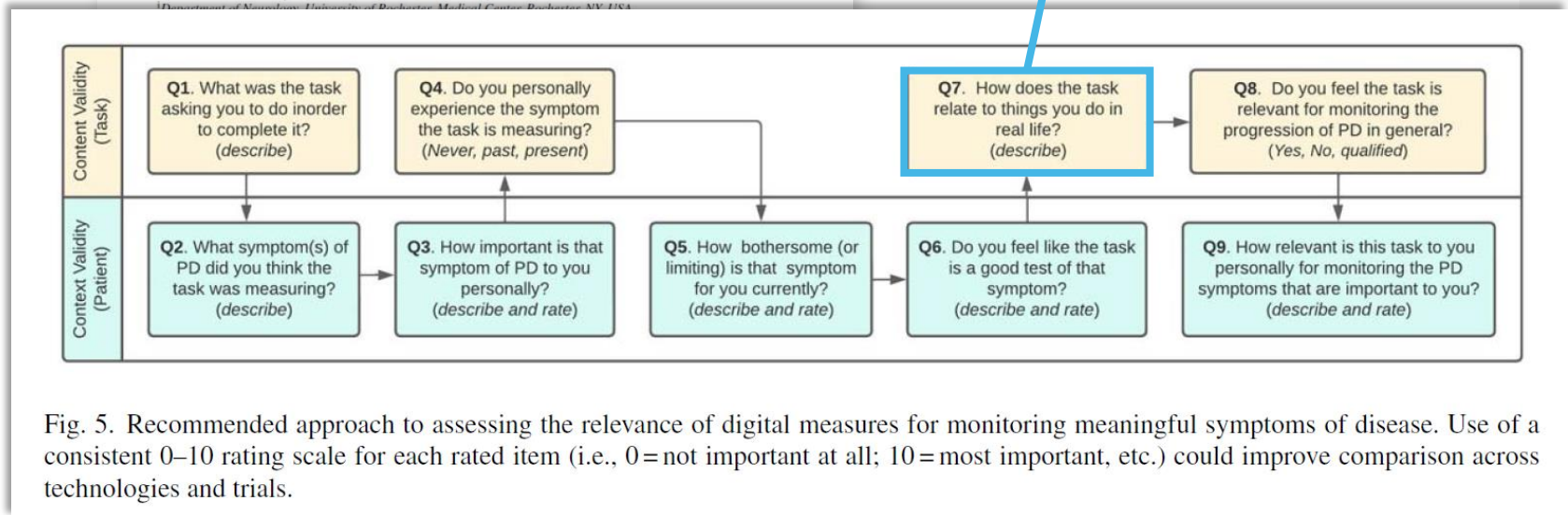


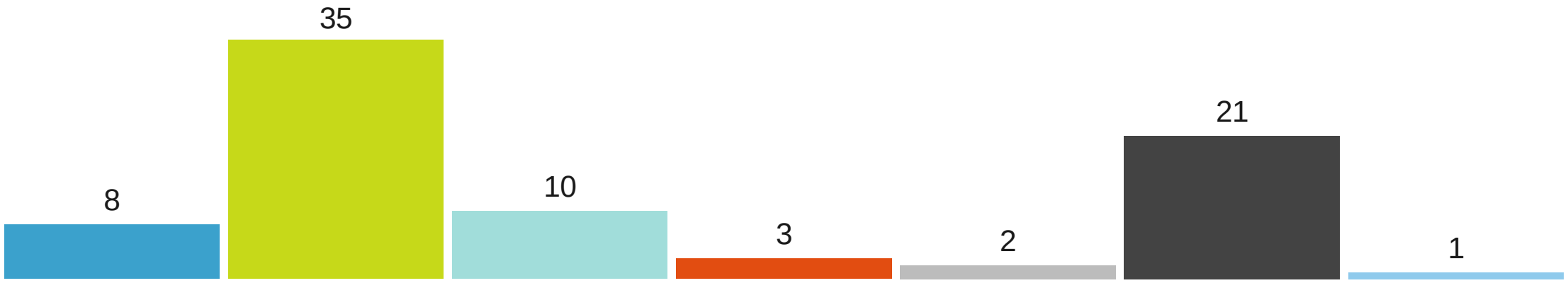
Fig. 5. Recommended approach to assessing the relevance of digital measures for monitoring meaningful symptoms of disease. Use of a consistent 0–10 rating scale for each rated item (i.e., 0 = not important at all; 10 = most important, etc.) could improve comparison across technologies and trials.

Finger tapping task

Participant performs rapid alternating finger movements by tapping two side-by-side targets using index and middle fingers.

Sensors enable a multitude of ways to derive outcome measures

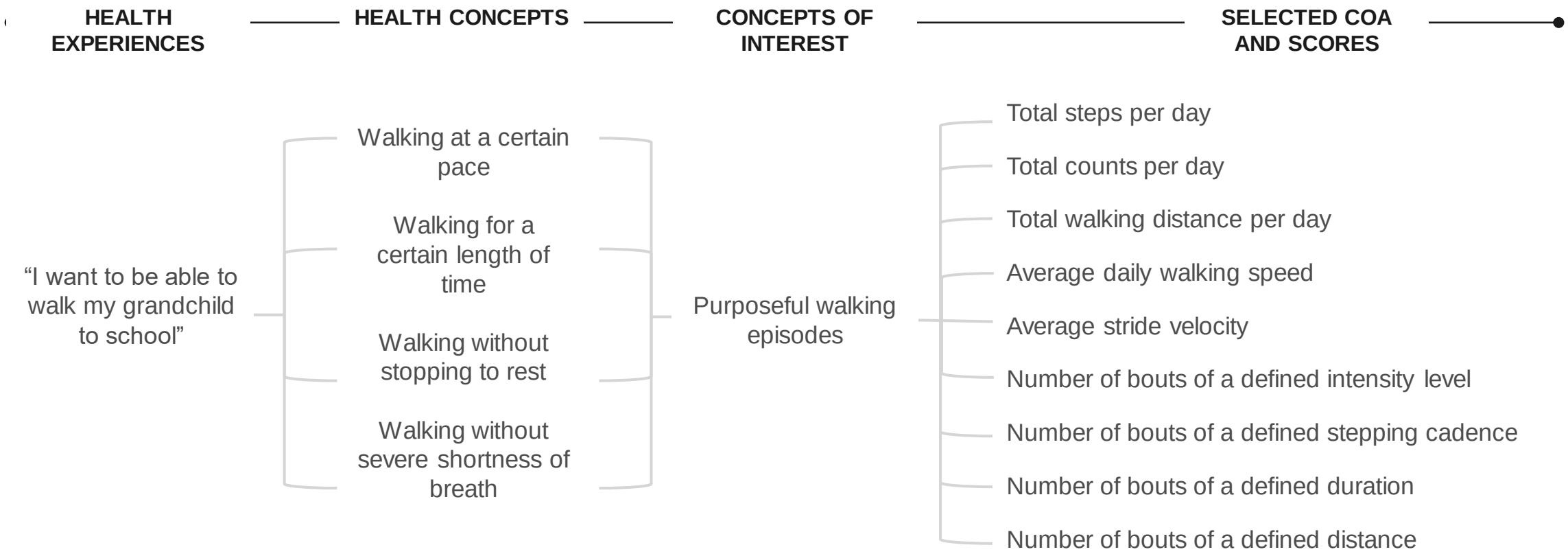
COPD: 76 studies examined, over 80 endpoints reported



Total activity	Time in bouts	Body position	Postural transitions	Time inactive/ active	Intensity	Diaurnal profile
• Total steps, counts, VMUs • MET·h • Energy expenditure • Distance walked	Bout thresholds defined based on: • Steps • Counts • METs • Walking speed	• Lying • Sitting • Standing • Walking • Total time • Maximum bout length	• Lie → stand • Sit → stand	• Time inactive • Time active	• Peak performance • PAL • Mean max 30 • Mean movement intensity (g) • Walking speed • #MVPA bouts • Bout intensity • Maximal METs • MVPA target achieved	• Hourly count profile

Byrom and Rowe. Contemp Clin Trials. 2016;47: 172-184.

Sensors enable a multitude of ways to derive outcome measures



Back to the Sysnav example…

Psychometric properties used to select SV95C from the five candidate outcome measures

- **Concurrent validity**: All stride parameters are consistent with 6MWT, NSAA and 4SC measures (n=45)
- **Known-groups discrimination**: Discriminate between controls and DMD accurately, especially when 95th percentile used
- **Sensitivity to change**: Assessed parameters at 6 and 12 months compared to 6MWT. 95th percentile shown more sensitive than the other measures – leads to greater statistical power and reduced sample sizes

	Sample size per group
50th percentile (median) stride length (m)	**202**
95th percentile stride length (m)	**53**
50th percentile (median) stride velocity (m/s)	**63**
95th percentile stride velocity (m/s)	**14**

Interpretability challenges

SV95C?

Statistically-derived composite measures?

- **More direct measures of concept of interest**
  - Interpretability may be a given (e.g. total steps per day)
- **More abstract / indirect measures**
  - Interpretation may be a greater challenge
  - Definition description by expert opinion / consensus
    - Develop layman interpretation
  - Interpretability through existing or new qualitative work

Conclusions

1. The traditional COA development process **may sometimes be a challenge** for the development of SBFOs

2. The end-result of evidencing **measurement of a meaningful aspect of health** remains vitally important, but there may be other valid approaches and points in the process to generate this

3. Sensors provide an array of possible measures, COA developers need to work with **digital health experts** to derive meaningful outcome measures

4. Statistical approaches to generate candidate outcome measures may be valid, but may be more **difficult to comprehend/interpret**

SBFO value beyond regulatory decision making

The value of sensor-based functional outcome measures for regulatory decision making

- Development of meaningful outcome measures to drive insights into treatment effects
- Providing real-world assessments within the confines of a clinical development program

Late phase (Payer and HTA Decision-Making)

- Measure SBFOs to explore how clinical trial efficacy maps to real-world effectiveness
- Value to payers and HTAs

Routine care (Physician and Patient Decision-Making)

- Wide-spread adoption in clinical trials and use in labelling may demonstrate the value of the same SBFO measures in routine care

SIGNANT HEALTH
THE EVIDENCE GENERATION COMPANY™

THANK YOU

bill.byrom@signanthealth.com

@billbyrom @signanthealth

How can sensor-based outcomes inform payer decision making?

Andrew Lloyd

DPhil, Acaster Lloyd Consulting Ltd.

AcasterLloyd
Consulting Ltd

Understanding value

Illustrative Example-
Congestive Heart Failure

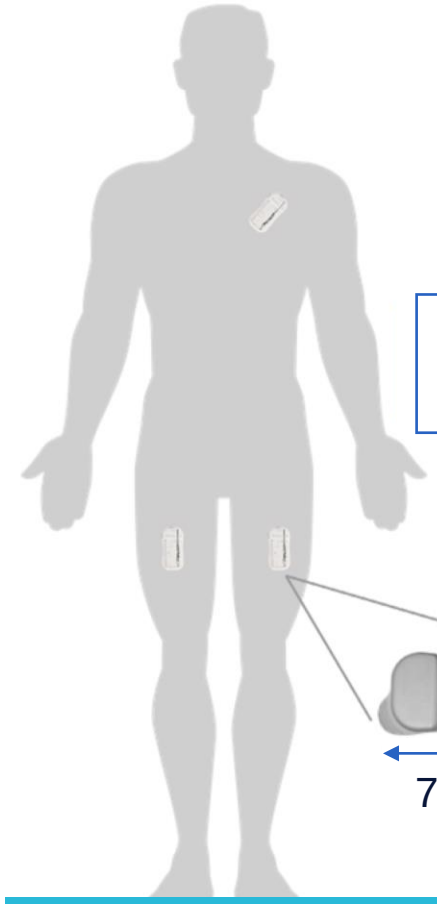
Concept Identification via Patient Interviews	
Walking grandchildren from school	
Do more for myself around the house	

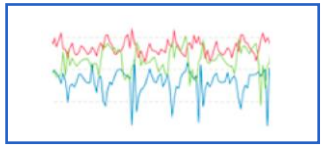
Outcomes Assessed via Sensors	
Time spent sitting/ lying down	Sit-to-stand transitions
Number of walking episodes	Time spent in walking episodes

Outcomes Relevant to Payers	
Health State Utility • EQ-5D • Minnesota Living with Health Failure (MLHF) Questionnaire	• Hospitalizations • Emergency room visits

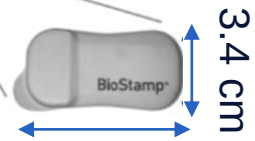
SBFOs: sensor-based functional outcomes

Sensor-based outcomes

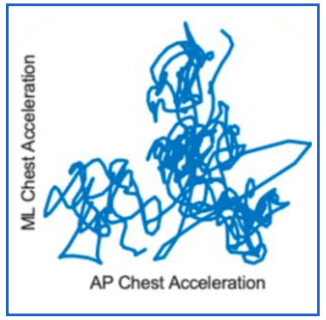




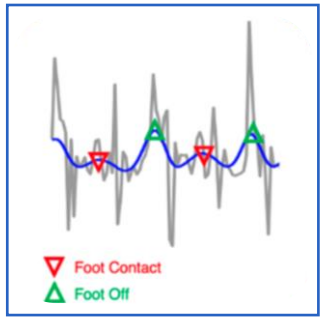
22 PwMS
for 6-weeks



Deep Learning
Activity
Classifier



Postural Sway



Gait

Difference testing

Level 1

Intra-class
Correlation

Level 2

PRM
Correlations

Level 3

Data Collection → **Activity Detection** → **Performance Measures** → **Wear Duration Analysis**

Meyer BM, etal. How Much Data Is Enough? A Reliable Methodology to Examine Long-Term Wearable Data Acquisition in Gait and Postural Sway. Sensors. 2022; 22(18):6982.

What can we measure?

Most commonly used to assess

- Physical activity, movement, sleep
- Physiological markers – heart rate, glucose etc

Sensor-based functional outcomes can measure events in real time

- Real time measurement is very hard using PROs
- Examples could include
  - *Measure a relapse in MS*
  - *Understand physical activity during chemotherapy*
  - *Assess shielding behaviour for immune compromised adults*

But there are challenges

- What is the actual meaning or value of changes in gait/postural sway (for example).
  - Does it inform us about prognosis?
- Trial sensors and real-world evidence
  - Can they be replaced by consumer devices for long term studies?
- Validation challenges – many published studies are very small

ISPOR elements of value

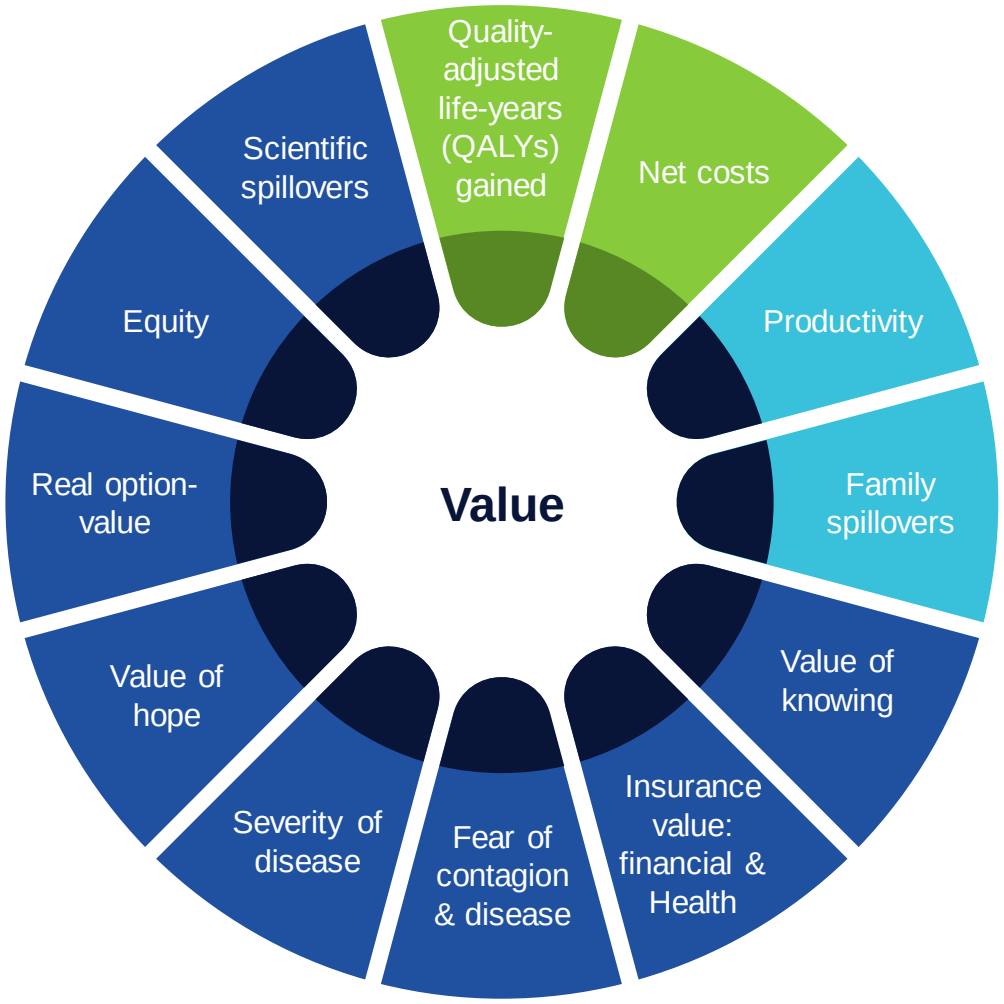
Where do sensor-based outcomes fit?

- Better understanding of outcomes?
- Translate efficacy to effectiveness?
- Reducing uncertainty

Elements of value from a patient perspective

- Payers value some other issues
- E.g. reducing risk & uncertainty

What value do sensor-based outcomes bring?



Supporting payers

Some experts suggest that one role of sensor-based outcomes is generating data for

- Improving our understanding of health care costs and how they could be managed
- Reducing costs of personalized care
- Building efficiencies
- Pay for performance or Value-based contracting

Internet of Medical Things

- Enables remote monitoring of patients
- Supports management of chronic disease such as diabetes, CVD
- Also supports pricing and insurance providers
- Availability of more data helps payers/insurers to better understand risks

But is this a limited view of the benefits of sensor-based outcomes?

HTA/Cost-effectiveness payers

HTA countries (Canada, UK, NL, Australia, Mexico etc)

- Focus on gains in health or HRQL measured using utility measures like EQ-5D
- Here HRQL is reduced to a single score 0 to 1 scale

HTA are interested in buying health

- Under a fixed health care budget – what's the biggest gain in health per $ spent?
- 'Stride velocity' or similar outcomes don't easily fit
- Work is needed to understand the value of such endpoints

EQ-5D ™ is a trade mark of the EuroQol Group

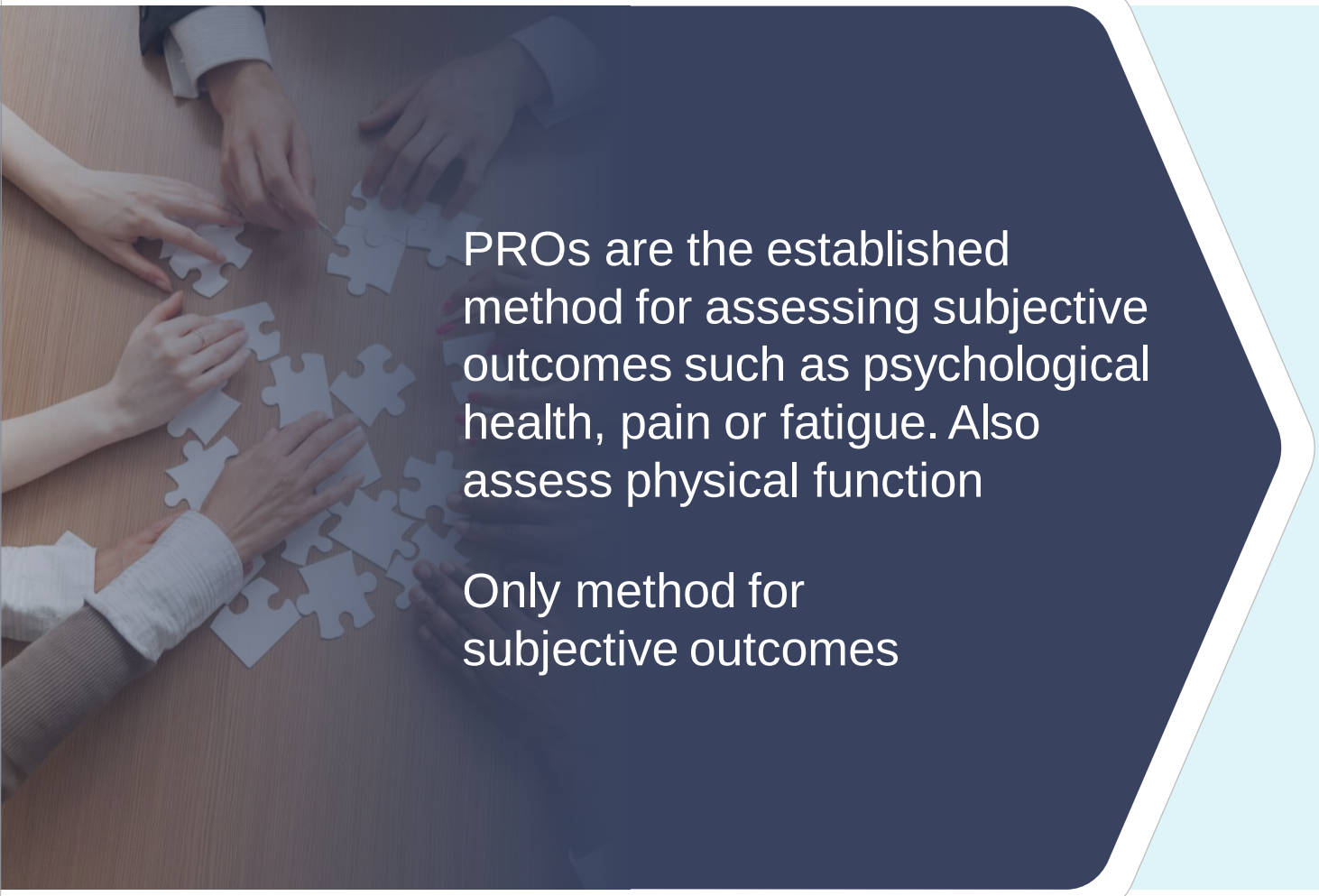
Image source: https://www.evidera.com/resource/health-technology-assessment-during-the-covid-19-pandemic/

Value of sensor based outcomes

abbvie

AcasterLloyd
Consulting Ltd

Problems with subjective measurement



PROs are the established method for assessing subjective outcomes such as psychological health, pain or fatigue. Also assess physical function

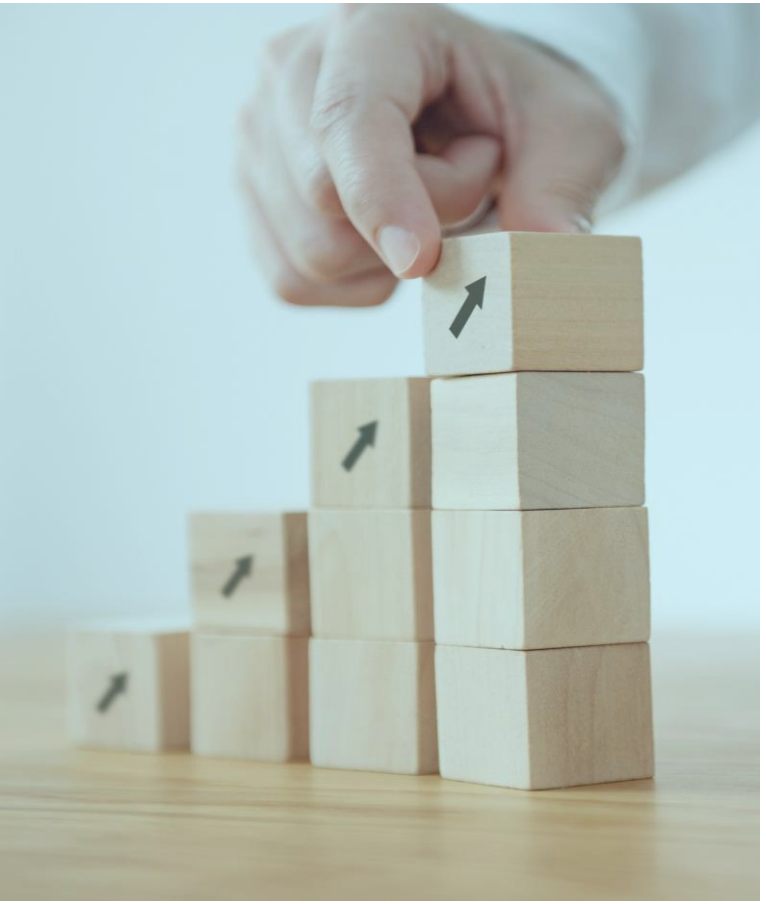
Only method for subjective outcomes

But consider the limitations of subjective data

- Subjective data can be noisy, biased, misinterpreted by patients
- Missing data is very common and not at random
- Relies on recall
- Difficult to interpret the meaning of changes in scores

Can sensor-based outcomes alongside PROs help to address these issues?

Some of the benefits of sensor-based outcomes



- Sensor data may provide more accurate measurement of some aspects of functioning

- Sensor-based outcomes may provide more certainty that trial outcomes translate into real world practice

- PRO data more likely to be missing for people who are more sick
  - Sensor-based outcomes may help us to avoid this

Challenges with sensor-based outcomes

Cost of implementation can be high

- Is the data worth the additional cost?
- To what extent can trial sensors be replaced with FitBits/ Apple Watch etc in a RW study

Interpretation of data

- What does a change in functional outcomes mean?
- Try to link to long term prognosis in a disease
- Or link sensor outcome to changes in what a patient can do

Validation of outcomes

- Literature full of small studies of ~20 people
- Need large validation studies to be able to draw inferences

Recommendations for payers

Recommend that payers align to provide guidance to industry regarding acceptability of digital endpoints & meaningful change

- *Getting payers to align is very challenging*
- *This could and should come from industry and expert bodies*

Payers should provide sponsors with early advice and engagement

- Engage early & often

But is this realistic?

- *Early may be possible, but frequent engagement not?*
- *HTA bodies are not ready for engagement on sensor based outcomes*
- *Many payers/ HTA will not provide a view on this*

https://www.dimesociety.org/wp-content/uploads/3P-considerations-and-recommendations.pdf

Sensor-based outcomes & payers

- This is a rapidly evolving area
- Industry and experts should work together to identify the challenges and issues to resolve
    - Validation
    - Interpretation and meaningfulness
    - Predictive/ prognostic value
- Development of guidance for payers regarding interpretation
    - Include payers/ HTA/experts/industry
- HTA/ payers need to be ready
    - Interpret and value sensor-based outcome data
    - For engagement with industry

Q & A

All presenters

PCOR

Patient-Centered
Outcomes Research

Polling questions for the audience to kick off Q&A

Option 1 (start with this question)—In your opinion, what is the biggest value driver for Sensor-Based Functional Outcome data from the payer perspective?

- Improving our understanding of health care costs and how they could be managed
- Reducing costs of personalized care
- Building efficiencies
- Pay for performance or Value based/Outcomes based contracting