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Patient-Centered Evidence (PCE) Consortium Update

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Clinical Outcome Assessment Program Annual Meeting
April 16-17, 2026
Washington, DC

Patient-Centered Evidence (PCE) Consortium

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- Formerly called the Patient-Reported Outcome (PRO) Consortium
- Formed in late 2008 by C-Path in cooperation with FDA's Center for Drug Evaluation and Research (CDER) and the pharmaceutical industry
- Membership
  - 25 members (pharmaceutical firms)
- Additional Participants
  - Representatives of governmental agencies (FDA, NIH)
  - Clinical consultants, patients, patient advocacy organizations, academic researchers, and contract research organizations partnering in the development of PRO measures and other COAs

PCE Consortium Members

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abbvie

AstraZeneca

BAYER

BIOMARIN

Boehringer Ingelheim

Bristol Myers Squibb™

Daiichi-Sankyo

EMD SERONO

Genentech
A Member of the Roche Group

GILEAD

GSK

Johnson&Johnson

Jazz Pharmaceuticals

Lilly

MERCK

NOVARTIS

novo nordisk®

Otsuka

Pfizer

REGENERON
SCIENCE TO MEDICINE®

sanofi

SHIONOGI

Takeda

ucb

ultragenyx

Patient-Centered Evidence (PCE) Consortium

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Positioning Statement

The Patient-Centered Evidence Consortium (PCEC) is a multi-stakeholder initiative dedicated to **advancing the development and use of patient-centered outcomes and clinical outcome assessments including those based on digital health technologies (DHTs) to derive endpoints that reflect what matters most to patients.** Through rigorous scientific methods, consensus-driven standards, and collaborative partnerships, PCEC transforms meaningful patient input into robust evidence that informs drug development, regulatory decisions, treatment access, and clinical decision making. By illuminating the path from outcomes to endpoints to evidence, PCEC ensures that patient perspectives play a central role in shaping better treatments and supporting informed choices for patients and healthcare providers.

Mission

To maximize impact of patient-centered evidence on drug development, regulatory, access, and treatment decision making, through rigorous scientific methods, standards, and multistakeholder collaboration.

Tagline

“Transforming patient-centered outcomes into evidence that drives decisions”

PCE Consortium's Objectives

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- Advance clinical outcome assessment (COA) and patient experience data (PED) measurement science through methodological innovation, data standardization, and harmonization initiatives.
- Promote the application of patient-centered evidence in regulatory, access, and treatment decision making through education, development and dissemination of industry best practices, and stakeholder engagement.
- Collaboratively develop fit-for-purpose COAs, inclusive of DHT-derived COAs, to address unmet measurement needs, pursue regulatory endorsement to support endpoints, and facilitate adoption to elevate the patient perspective in drug development and decision making.

Working Groups That Have Completed Their Initial Goal

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- IBS
- Asthma
- Non-Small Cell Lung Cancer
- Depression
- Myelofibrosis

Total licenses issued: 246

Diary for Irritable Bowel Syndrome Symptoms – Constipation (DIBSS-C)

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DIBSS-C

Asthma

Non-Small Cell Lung Cancer

Depression

Myelofibrosis

Obtained FDA qualification
December 18, 2020 – included in labeling by FDA

2 licenses for DIBSS-C
- 1 in phase 2b
- 1 not reported

For example, FDA expanded the label for the drug LINZESS® (linaclotide) to include results of a phase 3b clinical trial conducted by Ironwood Pharmaceuticals, Inc. and AbbVie Inc. that used DIBSS-C to assess an endpoint for improved abdominal symptoms (bloating, abdominal pain, abdominal discomfort).[6] This is the first time a PRO Consortium measure has been used to support a label claim.

Asthma Daytime Symptom Diary (ADSD)
Asthma Nighttime Symptom Diary (ANSD)

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Obtained FDA qualification
March 2019

30 licenses for ADSD and ANSD

- 1 in phase 1
- 5 in phase 2
- 1 in phase 2b
- 3 in phase 3
- 1 in phase 4
- 19 not reported

Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)

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Obtained FDA qualification
April 2018

124 licenses for NSCLC-SAQ

- 3 in phase 1
- 6 in phase 1/2
- 13 in phase 2
- 3 in phase 2/3
- 44 in phase 3
- 1 in phase 4
- 54 not reported

Meaningful change threshold estimation for the non-small cell lung cancer symptom assessment questionnaire (NSCLC-SAQ): psychometric analysis from a phase 3 trial (LIBRETTO-431)

Published: 23 January 2025

Volume 34, pages 1137–1146, (2025) Cite this article

Clarke, N., Worthy, G., Payakachat, N. *et al.* Meaningful change threshold estimation for the non-small cell lung cancer symptom assessment questionnaire (NSCLC-SAQ): psychometric analysis from a phase 3 trial (LIBRETTO-431). *Qual Life Res* **34**, 1137–1146 (2025). https://doi.org/10.1007/s11136-025-03895-1

Symptoms of Major Depressive Disorder Scale (SMDDS)

Obtained FDA qualification
November 2017

21 licenses for SMDDS

- 8 in phase 2
- 13 not reported

Myelofibrosis Symptom Assessment Form version 4.0 (MFSAF v4.0)

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IBS | Asthma | Non-Small Cell Lung Cancer | Depression | **MFSAF v4.0**

April 2017 - Derived the **consensus-defined MFSAF v4.0**
2018 – included in labeling by FDA, EMA, PMDA, Health Canada, and MHRA in 2023-24

68 licenses for *MFSAF v4.0*

- 2 in phase 1
- 1 in phase 1b
- 2 in phase 1/2
- 2 in phase 1b/2
- 8 in phase 2
- 11 in phase 3
- 1 in registry
- 41 not reported

Daskalopoulou, C., Gorsh, B., Dumi, G. *et al.* Myelofibrosis symptom assessment form total symptom score version 4.0: measurement properties from the MOMENTUM phase 3 study. *Qual Life Res* **34**, 739–750 (2025). https://doi.org/10.1007/s11136-024-03855-1

MFSAF v4.0 in Regulatory Approvals

Links to approvals from FDA, Health Canada, EMA, MHRA, and PMDA:

- FDA Approval
- EMA Approval
- PMDA Approval
- Health Canada Approval
- MHRA Approval

US. Food and Drug Administration (FDA), European Medicines Agency (EMA), Japan's Pharmaceutical and Medical Devices Agency (PMDA), Medicines and Healthcare products Regulatory Agency (MHRA)

Measures available for licensing

These measures are actively being licensed for use in clinical trials via the following website: https://www.c-pathcoas.org/

ASTHMA
- Asthma Daytime Symptom Diary (ADSD) and the Asthma Nighttime Symptom Diary (ANSD)

FUNCTIONAL DYSPEPSIA
- Functional Dyspepsia Symptom Diary (FDSD)

IRRITABLE BOWEL SYNDROME
- Diary for Irritable Bowel Syndrome Symptoms - Constipation (DIBSS-C)
- Diary for Irritable Bowel Syndrome Symptoms - Mixed (DIBSS-M)

MAJOR DEPRESSIVE DISORDER
- Symptoms of Major Depressive Disorder Scale (SMDDS)

MYELOFIBROSIS
- Myelofibrosis Symptom Assessment Form v4.0 (MFSAF v4.0) Diary
- Myelofibrosis Symptom Assessment Form v4.0 (MFSAF v4.0) 7-day Recall

NON-SMALL CELL LUNG CANCER
- Non-Small ell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)

New Consortium Efforts

New and Upcoming Projects

- **Working group launched in 2025:** Engaging Sponsor Stakeholders on the Value of COAs
  - Helping sponsors strengthen how COA data are used in development and access evidence generation strategies.
    - Overview of the Development and Validation of a Fit-for- Purpose PRO Measure
    - Site and patient experience and perceived burden with PRO measures
    - Resources to highlight the importance of early planning to maximize opportunity for success
    - Resources to support COA Dossier development
- **Working group launched in 2026:** engagement with regulatory stakeholders.
- **Working group** on engagement with value assessment stakeholders coming soon!

Ongoing Consortium Efforts

Goal of Current Working Group Projects

To produce and/or compile the necessary evidence to enable new or existing COAs to be qualified by FDA for use in clinical trials where COA-based endpoints can be used to support product labeling claims.

- 8 therapeutic area working groups
- 13 COAs in FDA COA Qualification Program

Cognition in Alzheimer's disease (1 PerfO measure)	Chronic Heart Failure (2 PRO measures + DHT-based COA)	Depression (2 PRO measures)	Irritable Bowel Syndrome (1 PRO measure)
Multiple Sclerosis (2 PRO measures)	Pediatric Asthma (1 PRO and 1 ObsRO measure)	Rheumatoid Arthritis (1 PRO measure)	Small Cell Lung Cancer (1 PRO measure)

Chronic Heart Failure (CHF)

1/5

Working toward qualification of an activity monitor-based endpoint measure of physical activity and two PRO measures developed by Amgen

- Chronic Heart Failure-Symptom Scale (CHF-SS)
- Chronic Heart Failure-Impact Scale (CHF-IS)

  Context of use: adults with a clinician-confirmed history of CHF for ≥3 months with New York Heart Association class II to IV symptoms for ≥4 weeks, with preserved ejection fraction (HFpEF) or with reduced ejection fraction (HFrEF)

Since April 2025…

Q1 2024	Q4 2024	Q4 2025
CHF-IS QP submission to FDA March 20, 2024 **FDA reviewability assessment in progress**	Step count-based measure QP submission to FDA December 18, 2024 **FDA reviewability assessment in progress**	**CHF-SS QP** **Accepted by FDA** **November 15, 2025**

Depression WG 2.0

2/5

Working toward qualification of

- Symptoms of Major Depressive Disorder Diary (SMDDD)
- Symptoms of Major Depressive Disorder Momentary Assessment (SMDDMA)

  Context of use: Persons 18 years and older with a diagnosis of major depressive disorder, who are being treated in ambulatory settings with rapid-acting antidepressants

Since April 2025…

SMDDMA QP submission to FDA

March 24, 2023

FDA reviewability assessment in progress

Q1 2023

SMDDD QP

Accepted by FDA

September 23, 2024

Q3 2024

SMDDD qualitative study in progress

Startup is underway

Q1 2026

Irritable Bowel Syndrome (IBS)

3/5

Working toward qualification of

- Diary for Irritable Bowel Syndrome Symptoms – Diarrhea (DIBSS-D)

  Context of use: Adults diagnosed with IBS - diarrhea predominant (IBS-D)

Since April 2025…

DIBSS-D QP **accepted by FDA**

January 8, 2024

Q1 2024

DIBSS-D FQP **submission to FDA**

Target submission

Goal: Q2 2026

Multiple Sclerosis (MS)

4/5

Working toward qualification of

- PROMIS® Short Form v1.0—Fatigue-Multiple Sclerosis 8a (PROMIS FatigueMS—8a)
- PROMIS®/Neuro-QoL™ Physical Function Measure for Multiple Sclerosis (PROMISnq Short Form v2.0 - Physical Function - Multiple Sclerosis 15a [PROMISnq PFMS—15a])

  Context of use: Adults with any type of MS

Since April 2025…

PROMISnq PFMS—15a QP **Accepted by FDA** November 3, 2023 — Q4 2023

Qualitative study of anchors of fatigue and physical function completed — Q1 2025

PROMIS FatigueMS—8a FQP **submission to FDA** July 24, 2025 — Q3 2025

PROMISnq PFMS—15a FQP **submission to FDA** February 4, 2026 — Q1 2026

Rheumatoid Arthritis (RA)

5/5

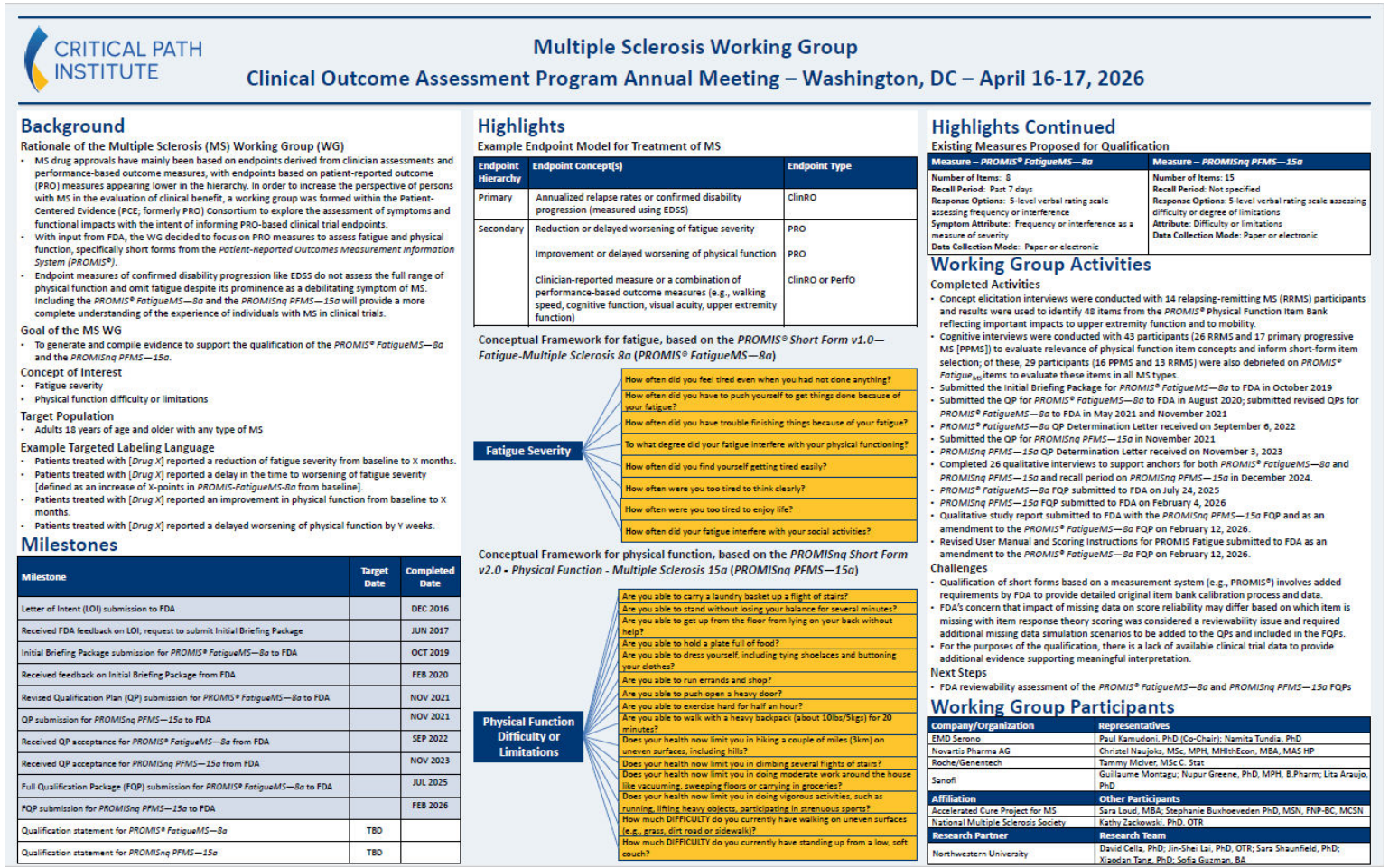
Working toward qualification of PROMIS® Fatigue Short Form 10a

Context of use: adults diagnosed with RA

Since April 2025…

Working Group Posters

See working group posters for more detailed information in CVENT meeting portal



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Multiple Sclerosis Working Group

Clinical Outcome Assessment Program Annual Meeting – Washington, DC – April 16-17, 2026

Background

Rationale of the Multiple Sclerosis (MS) Working Group (WG)

- MS drug approvals have mainly been based on endpoints derived from clinician assessments and performance-based outcome measures, with endpoints based on patient-reported outcome (PRO) measures appearing lower in the hierarchy. In order to increase the perspective of persons with MS in the evaluation of clinical benefit, a working group was formed within the Patient-Centered Evidence (PCE; formerly PRO) Consortium to explore the assessment of symptoms and functional impacts with the intent of informing PRO-based clinical trial endpoints.
- With input from FDA, the WG decided to focus on PRO measures to assess fatigue and physical function, specifically short forms from the *Patient-Reported Outcomes Measurement Information System (PROMIS®)*.
- Endpoint measures of confirmed disability progression like EDSS do not assess the full range of physical function and omit fatigue despite its prominence as a debilitating symptom of MS. Including the *PROMIS® FatigueMS—8a* and the *PROMISnq PFMS—15a* will provide a more complete understanding of the experience of individuals with MS in clinical trials.

Goal of the MS WG

- To generate and compile evidence to support the qualification of the *PROMIS® FatigueMS—8a* and the *PROMISnq PFMS—15a*.

Concept of Interest

- Fatigue severity
- Physical function difficulty or limitations

Target Population

- Adults 18 years of age and older with any type of MS

Example Targeted Labeling Language

- Patients treated with [*Drug X*] reported a reduction of fatigue severity from baseline to X months.
- Patients treated with [*Drug X*] reported a delay in the time to worsening of fatigue severity [defined as an increase of X-points in *PROMIS-FatigueMS-8a* from baseline].
- Patients treated with [*Drug X*] reported an improvement in physical function from baseline to X months.
- Patients treated with [*Drug X*] reported a delayed worsening of physical function by Y weeks.

Milestones

Milestone	Target Date	Completed Date
Letter of Intent (LOI) submission to FDA		DEC 2016
Received FDA feedback on LOI; request to submit Initial Briefing Package		JUN 2017
Initial Briefing Package submission for *PROMIS® FatigueMS—8a* to FDA		OCT 2019
Received feedback on Initial Briefing Package from FDA		FEB 2020
Revised Qualification Plan (QP) submission for *PROMIS® FatigueMS—8a* to FDA		NOV 2021
QP submission for *PROMISnq PFMS—15a* to FDA		NOV 2021
Received QP acceptance for *PROMIS® FatigueMS—8a* from FDA		SEP 2022
Received QP acceptance for *PROMISnq PFMS—15a* from FDA		NOV 2023
Full Qualification Package (FQP) submission for *PROMIS® FatigueMS—8a* to FDA		JUL 2025
FQP submission for *PROMISnq PFMS—15a* to FDA		FEB 2026
Qualification statement for *PROMIS® FatigueMS—8a*	TBD	
Qualification statement for *PROMISnq PFMS—15a*	TBD	

Highlights

Example Endpoint Model for Treatment of MS

Endpoint Hierarchy	Endpoint Concept(s)	Endpoint Type
Primary	Annualized relapse rates or confirmed disability progression (measured using EDSS)	ClinRO
Secondary	Reduction or delayed worsening of fatigue severity	PRO
	Improvement or delayed worsening of physical function	PRO
	Clinician-reported measure or a combination of performance-based outcome measures (e.g., walking speed, cognitive function, visual acuity, upper extremity function)	ClinRO or PerfO

Conceptual Framework for fatigue, based on the *PROMIS® Short Form v1.0—Fatigue-Multiple Sclerosis 8a (PROMIS® FatigueMS—8a)*

Conceptual Framework for physical function, based on the *PROMISnq Short Form v2.0 - Physical Function - Multiple Sclerosis 15a (PROMISnq PFMS—15a)*

Highlights Continued

Existing Measures Proposed for Qualification

Measure – *PROMIS® FatigueMS—8a*	Measure – *PROMISnq PFMS—15a*
Number of Items: 8 **Recall Period:** Past 7 days **Response Options:** 5-level verbal rating scale assessing frequency or interference **Symptom Attribute:** Frequency or interference as a measure of severity **Data Collection Mode:** Paper or electronic	**Number of Items:** 15 **Recall Period:** Not specified **Response Options:** 5-level verbal rating scale assessing difficulty or degree of limitations **Attribute:** Difficulty or limitations **Data Collection Mode:** Paper or electronic

Working Group Activities

Completed Activities

- Concept elicitation interviews were conducted with 14 relapsing-remitting MS (RRMS) participants and results were used to identify 48 items from the *PROMIS®* Physical Function item Bank reflecting important impacts to upper extremity function and to mobility.
- Cognitive interviews were conducted with 43 participants (26 RRMS and 17 primary progressive MS [PPMS]) to evaluate relevance of physical function item concepts and inform short-form item selection; of these, 29 participants (16 PPMS and 13 RRMS) were also debriefed on *PROMIS® Fatigue$_{MS}$* items to evaluate these items in all MS types.
- Submitted the Initial Briefing Package for *PROMIS® FatigueMS—8a* to FDA in October 2019
- Submitted the QP for *PROMIS® FatigueMS—8a* to FDA in August 2020; submitted revised QPs for *PROMIS® FatigueMS—8a* to FDA in May 2021 and November 2021
- *PROMIS® FatigueMS—8a* QP Determination Letter received on September 6, 2022
- Submitted the QP for *PROMISnq PFMS—15a* in November 2021
- *PROMISnq PFMS—15a* QP Determination Letter received on November 3, 2023
- Completed 26 qualitative interviews to support anchors for both *PROMIS® FatigueMS—8a* and *PROMISnq PFMS—15a* and recall period on *PROMISnq PFMS—15a* in December 2024.
- *PROMIS® FatigueMS—8a* FQP submitted to FDA on July 24, 2025
- *PROMISnq PFMS—15a* FQP submitted to FDA on February 4, 2026
- Qualitative study report submitted to FDA with the *PROMISnq PFMS—15a* FQP and as an amendment to the *PROMIS® FatigueMS—8a* FQP on February 12, 2026.
- Revised User Manual and Scoring Instructions for PROMIS Fatigue submitted to FDA as an amendment to the *PROMIS® FatigueMS—8a* FQP on February 12, 2026.

Challenges

- Qualification of short forms based on a measurement system (e.g., PROMIS®) involves added requirements by FDA to provide detailed original item bank calibration process and data.
- FDA's concern that impact of missing data on score reliability may differ based on which item is missing with item response theory scoring was considered a reviewability issue and required additional missing data simulation scenarios to be added to the QPs and included in the FQPs.
- For the purposes of the qualification, there is a lack of available clinical trial data to provide additional evidence supporting meaningful interpretation.

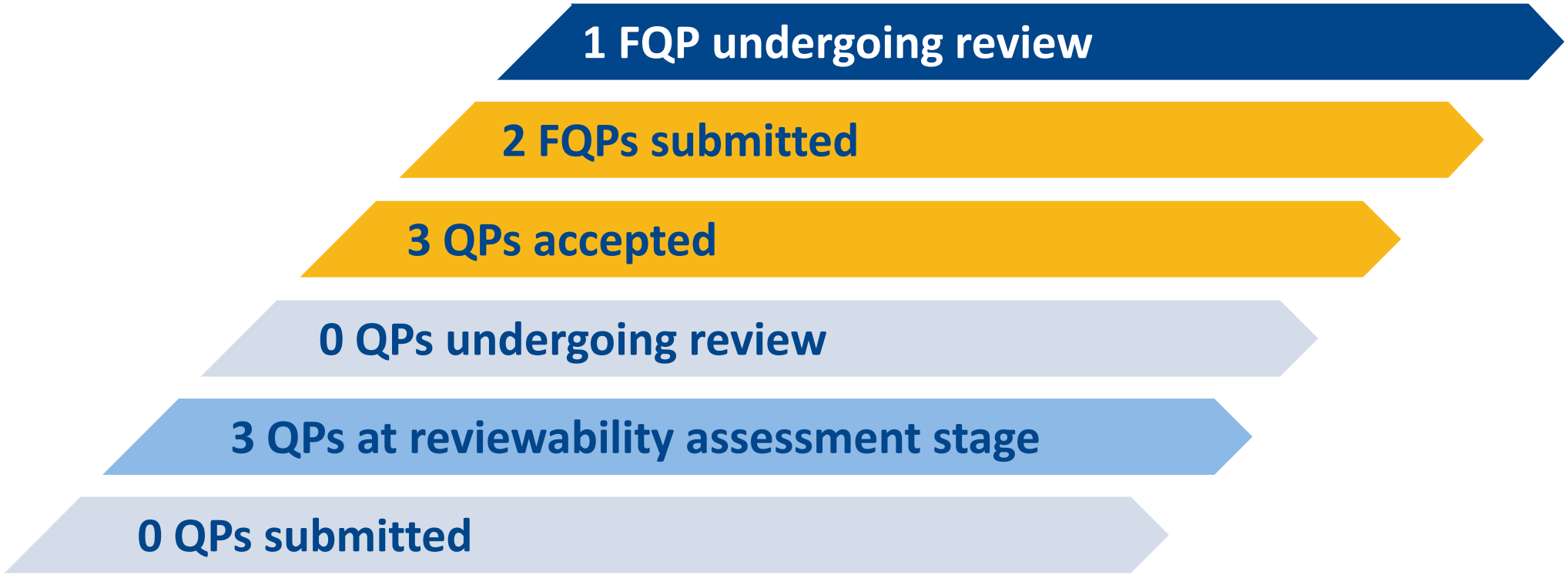
Next Steps

- FDA reviewability assessment of the *PROMIS® FatigueMS—8a* and *PROMISnq PFMS—15a* FQPs

Working Group Participants

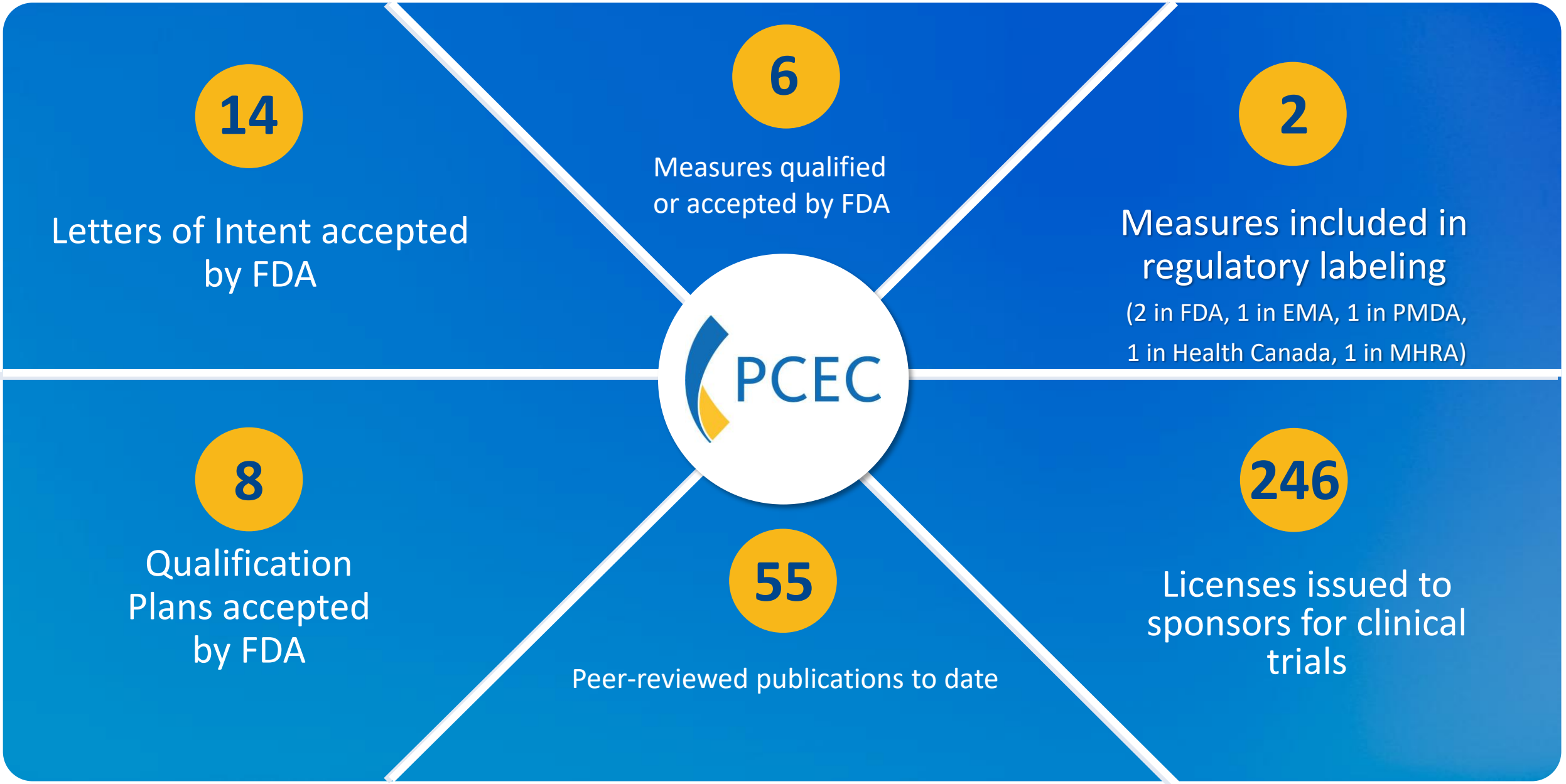
Company/Organization	Representatives
EMD Serono	Paul Kamudoni, PhD (Co-Chair); Namita Tundia, PhD
Novartis Pharma AG	Christel Naujoks, MSc, MPH, MHlthEcon, MBA, MAS HP
Roche/Genentech	Tammy Melver, MSc C. Stat
Sanofi	Guillaume Montagu; Nupur Greene, PhD, MPH, B.Pharm; Lita Araujo, PhD
Affiliation	**Other Participants**
Accelerated Cure Project for MS	Sara Loud, MBA; Stephanie Buxhoeveden PhD, MSN, FNP-BC, MCSN
National Multiple Sclerosis Society	Kathy Zackowski, PhD, OTR
Research Partner	**Research Team**
Northwestern University	David Cella, PhD; Jin-Shei Lai, PhD, OTR; Sara Shaunfield, PhD; Xiaodan Tang, PhD; Sofia Guzman, BA

Summary of PCE Consortium Qualification Submission Status for 2025



Publications since April 2025

Methods	• Considerations and Approaches to Establishing Estimates of Meaningful Change for Digital Endpoints as Drug Development Tools - *Therapeutic Innovation & Regulatory Science*
Best Practices	• Principles of Good Practice for Translation of Electronic Clinical Outcome Assessments - *Journal of Patient-Reported Outcomes* • Principles of Good Practice for Concept Definition in the Context of Translation and Linguistic Validation of Clinical Outcome Assessments (COAs) - *Journal of Patient-Reported Outcomes* • SISAQOL-IMI Consensus-based Guidelines to Design, Analyse, Interpret and Present Patient-reported Outcomes in Cancer Clinical Trials - *The Lancet Oncology* • Best Practice Recommendations and Considerations for Designing and Electronically Implementing Event-driven Diaries in Clinical Trials – *Nature Partner Journals Digital Medicine* • Use of AI within COA Linguistic Validation and eCOA Migration Processes: Analysis and Good Practice Recommendations - *Journal of Patient-Reported Outcomes*
Reviews	• Defining Score Interpretation Thresholds for Clinical Outcome Assessments: A Review of Terminology and Reporting Recommendations - *Journal of Patient-Reported Outcomes*



View Publications/White Papers/GBTI* Resources Library for the complete list of peer-reviewed publications to date.

U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA), Japan's Pharmaceutical and Medical Devices Agency (PMDA), Medicines and Healthcare products Regulatory Agency (MHRA)

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Thank you
for your support and partnership!

Join us on the journey to achieve our goals for 2026 and beyond!

https://c-path.org/program/patient-centered-evidence-consortium/