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Using patient experience data to inform value assessment for access decision making

Clinical Outcome Assessment Program Annual Meeting
April 16-17, 2026
Washington, D.C.

Disclaimer

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Session Participants

- **Moderator**
  - *Ebony Dasheill-Aje, PhD* – Executive Director & Head, Patient Centered Outcomes Science, BioMarin Pharmaceutical Inc.
- **Presenters**
  - *Jessica Abel, MPH* – Director, PED Policy & Best Practices, AbbVie
  - *Iyar Mazar, PhD* – Director, Patient-Centered Outcomes Assessment, Pfizer
  - *Leah Howard, JD* – President and Chief Executive Officer, National Psoriasis Foundation
- **Additional Panelist**
  - *Dana McCormick, RPh, FAMCP* – Senior Director, Practice Strategy & Innovation, AMCP

Session Agenda

- Overview of session (5 minutes)
- Presentations and remarks (33 minutes)
- Panel discussion (25 minutes)
- Open discussion and Q&A (12 minutes)

Session Objectives

This session will examine how patient experience data (PED) is being integrated into health technology assessment (HTA) and payer decision-making processes. Topics include:

- Reviewing HTA archetypes and the current landscape regarding varying requirements for use of PED
- Understanding US payer awareness and readiness to incorporate PED into formulary and coverage decisions
- Examining real-world case studies of successful patient advocacy organization engagement with ICER and payers
- Identifying opportunities and challenges for sponsors in generating and presenting PED to support value demonstration
- Discussing best practices for meaningful integration of PED and patient-centered evidence into value assessments

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Landscape

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Does One-Size-Fit-All? Navigating HTA Expectations for PED

Jessica Abel, MPH
Director, PED Policy & Best Practices
AbbVie

Patient Experience Data (PED)

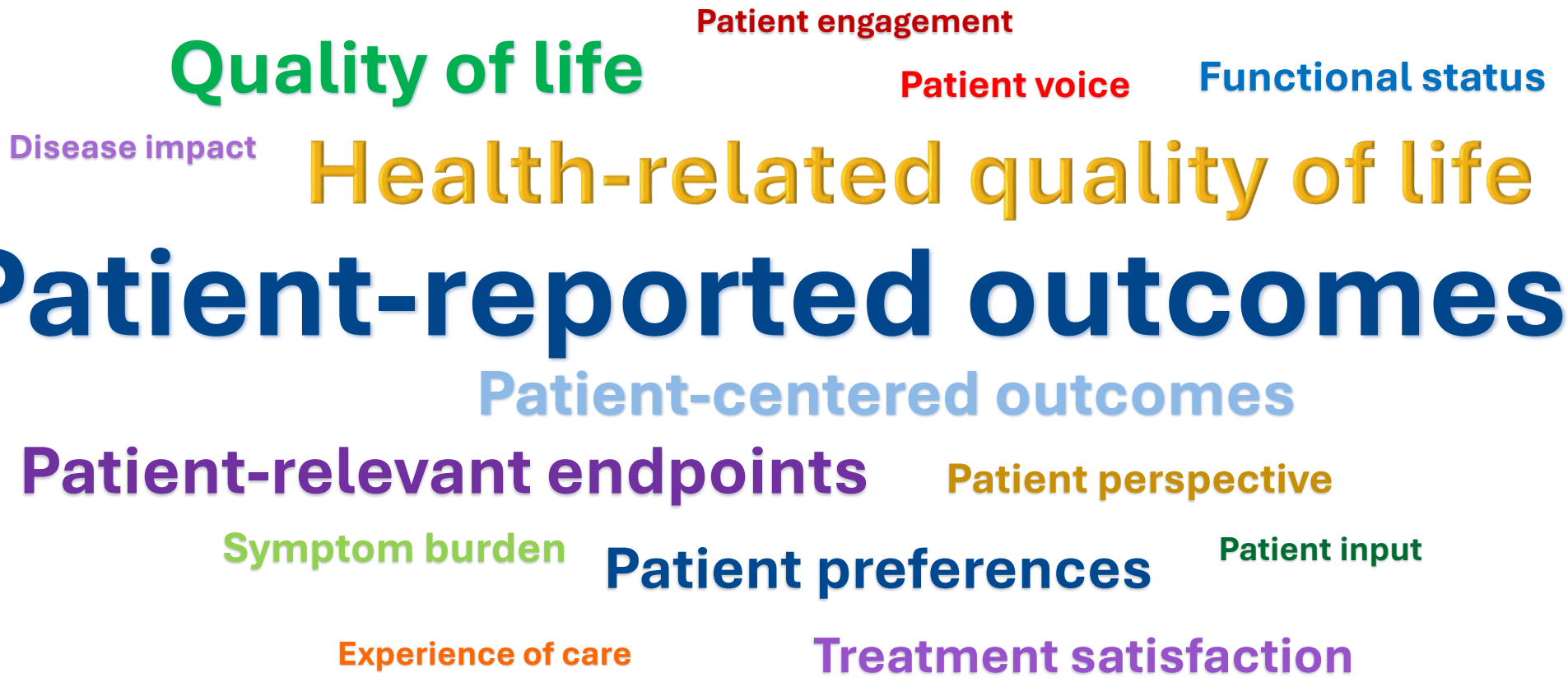
US Definition*: Data that are collected by any persons and are intended to provide information about patients' experiences with a disease or condition. PED can be interpreted as information that captures patients' experiences, perspectives, needs, and priorities related to (but not limited to):

1) The symptoms of their condition and its natural history;
2) The impact of the condition on their functioning and quality of life;
3) Their experience with treatments;
4) Input on which outcomes are important to them;
5) Patient preferences for outcomes and treatments; and
6) The relative importance of any issue as defined by patients.

EU Definition**: Data that directly reflect the experience of a patient or carer, without input or interpretation by a healthcare professional, third party or (artificial intelligence-based) device. Patient experience can include, but is not limited to, health and functional status, disease symptoms, disease course, treatment preferences, QOL, factors impacting treatment adherence, treatment outcomes and side effects.

* Defined in Title III, section 3001 of the 21st Century Cures Act, as amended by section 605 of the FDA Reauthorization Act of 2017 (FDARA)
** Defined in draft EMA Reflection Paper on PED, Sept 2025

Variable Terminology Used to Describe PED in HTA



Two (or Three?) Global HTA Archetypes

- HTA agencies evaluate clinical, economic & patient-focused evidence to inform policy decisions on coverage, pricing & reimbursement

Comparative effectiveness assessment (CEA)	CEA plus economic evidence (CEA+EE)	Combination of CEA & CEA+EE
Germany	**UK, France, Italy, Spain, Canada, Australia**	**US**

• Focuses on evaluating changes in patient-relevant endpoints to fully assess how a patient feels, functions, & survives
• Key components assessed:
  • Mortality
  • Morbidity (symptoms, clinical events)
  • HRQoL (disease-specific & generic)
  • Safety & tolerability
	• QALY is a key parameter to assess 'value for money'
	• Economic models use health state utilities to obtain ICER
	• HTA agencies use ICER to inform reimbursement & pricing decisions
	• In France, clinical added value assessments also include review of HRQoL data
		• No national HTA body
		• HTA & value assessment conducted by a mix of organizations using CEA or CEA+EE, including:
		  • Government entities (e.g., CMS, states)
		  • Private payers, PBMs
		  • Employers
		  • Institute for Clinical & Economic Review and other value assessors
		  • Biopharma company formulary dossiers

CEA, comparative effectiveness assessment; CMS, Centers for Medicare & Medicaid Services; EE, economic evidence; HTA, health technology assessment; ICER, incremental cost-effectiveness ratio; PBMs, Pharmacy Benefit Managers; QALY, quality-adjusted life year; HRQoL, health-related quality of life, UK, United Kingdom; US, United States

Do HTA Agencies Value PED as Central to Decision-Making?

- Variation exists by archetype in PED expectations & requirements for decision-making

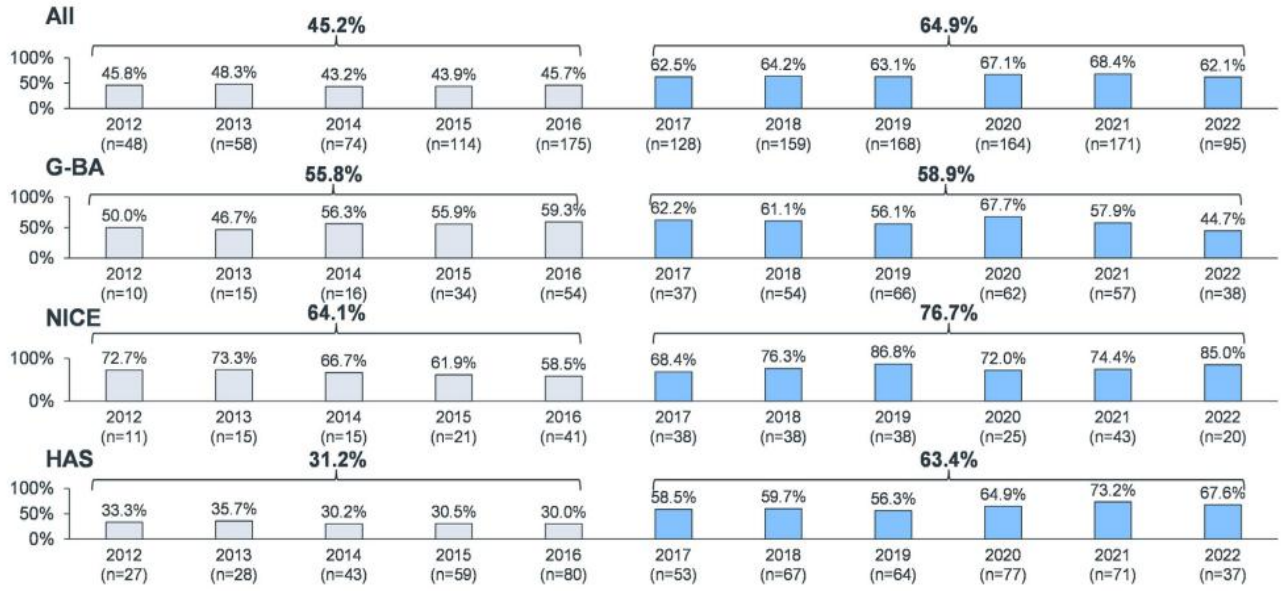
Comparative effectiveness assessment (CEA)	CEA plus economic evidence (CEA+EE)	Combination of CEA and CEA+EE
Germany	**UK, France, Italy, Spain, Canada, Australia**	**US**
• PED ***required*** for clinical assessments • HRQoL data mandated by German law for decision-making • Strict requirements for COA-based endpoints (pre-specified, validated COAs, minimal missing data) • Additional benefit rating requires clinically meaningful within-patient change responder threshold to be ≥15% of the scale range	• **UK/NICE**: PED ***valued*** & integrated in economic evaluations; PED used for cost-effectiveness models; HRQoL utilities inform economic evaluations • **France/HAS**: PED ***valued*** & can enhance clinical added value; HRQoL/PRO impact highly valued—not always required but absence may impact value score; strict evidence criteria (validated COAs, robust methods) • **Canada/CDA:** PED ***optional,*** considered case-by-case; standards for evidence & impact vary	• Payer interest in PED increasing, but expectations & evidentiary requirements are unclear • PED seen as useful for supplementing clinical trial data but limited familiarity restricts its utility in decision-making

CEA, comparative effectiveness assessment; CDA, Canada's Drug Agency; EE, economic evidence; HAS, Haute Autorité de santé; HRQoL, health-related quality of life; HTA, health technology assessment; NICE, National Institute for Health and Care Excellence; UK, United Kingdom; US, United States

Kanu C., Abel J.L.,Knight S.L. et al. J Patient Rep Outcomes (2026).

Increasing Use of PED for HTA Decision-Making

Inclusion of nonmandatory COAs* in HTA from 2012 to 2022



G-BA indicates The German Federal Joint Committee; HAS, French Haute Autorité de Santé; NICE, National Institute for Health and Care Excellence.

*Nonmandatory COAs include COAs beyond those required for assessing primary endpoints or determining cost-effectiveness to support regulatory or market access approval

- Number of nonmandatory COAs in HTA across G-BA, NICE, and HAS increased from 45.2% to 64.9% over a 10-year period
- Positive trends across all 3 HTA bodies reflect increased recognition of added value of PED, inclusive of COAs, in HTA decision-making

Krol et al. Value Health. 2025 Aug;28(8):1195-1203.

COA– Clinical outcome assessment; G-BA– Gemeinsamer Bundesausschuss; HAS– Haute Autorité de Santé; HTA– Health technology assessment; NICE– National Institute for Health and Care Excellence; PED– Patient experience data

Global PED Strategies Must Start with the End in Mind

For optimal approval, quality access, and maximum adoption, all drug development hurdles must be considered in a comprehensive PED strategy

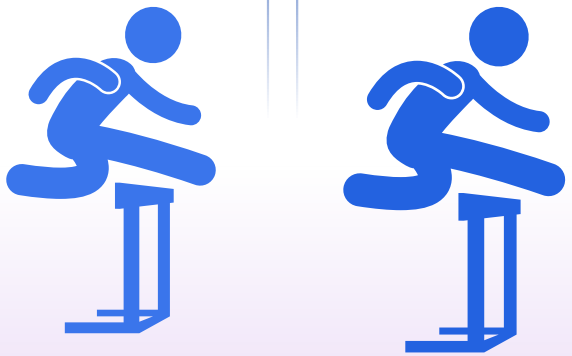
Optimal Approval

Drug Licensing/ Regulatory Approval

Safety | Efficacy | Quality

Quality Access

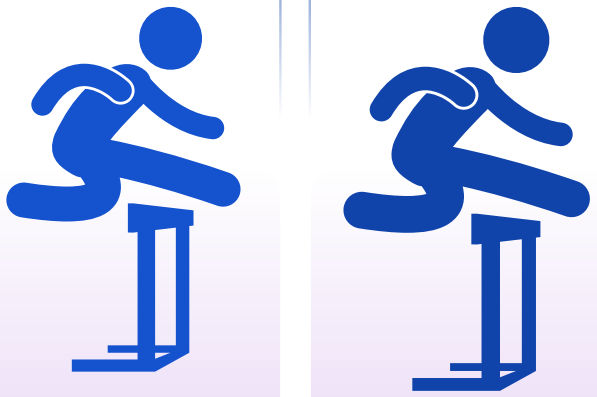
Reimbursement/ National Market Access | Local Market Access



Payers, Healthcare Providers, Patients and Patient Advocates

Adoption

Prescribing New Medicine | Adherence & Persistence with Medication



Physicians | Patients

Opportunities Exist to Enhance PED Strategies & Evidence Generation to Support Access Decision-Making

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PED Strategy

- Consider both access & regulatory needs when identifying concepts, measures, & endpoints
- Ensure PED reflects concepts important to the target patient population
- Consider collecting data on burden of illness, unmet need & patient preferences to complement COA data from clinical trials

Clinical Trial Design

- Ensure data quality for HTA-relevant trial endpoints (minimal missing) & appropriate placement in the testing hierarchy (i.e., alpha-controlled)
- Consider value of in-trial interviews (particularly to collect data to address potential unblinding concerns in open-label trials)

Sponsor Submissions

- Maximize use of PED—include qualitative data and patient preferences to complement trial data
- Align to agency guidance & ensure local affiliates understand value of including PED
- Highlight supportive PED evidence in executive summaries for reviewers

There is No One-Size-Fits-All Approach

- **HTA PED evidence standards vary by archetype & country**
  - **Germany:** Robust PED requirements; HRQoL/PRO data must meet strict criteria
  - **UK, France, Canada:** PED valued but evidentiary standards and impact vary
- **Global harmonization needed on terminology & evidentiary standards for PED** to facilitate efficient evidence generation & enhance impact of PED in access decision-making
- **Global PED strategies should be designed with access stakeholders in mind** to ensure the right evidence is available & integrated early and throughout a development program to inform decision-making

Thank You!

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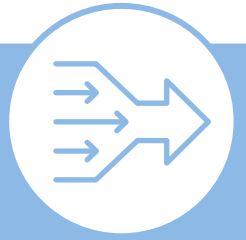
US Payers and Patient Experience Data: Where Do We Stand?

Iyar Mazar, PhD
Patient-Centered Outcomes Assessment (PCOA)
Pfizer

Why Are We Talking About US Payers?

Most of us here are **experts in patient-centered outcomes research** who have focused on generating evidence for regulatory review

Are we **optimizing Patient Experience Data (PED) so that patients are getting access** to the interventions that they value most?



Go beyond registration

- Identify & bridge PED knowledge gaps
- Advocate for PED in access decision-making
- Partner with payers on how to use PED

AMCP Patient Experience Data Survey

- Survey of **Academy of Managed Care Pharmacy (AMCP) members** to explore understanding and use of PED in formulary decision-making
- **60 completed responses** (6,960 emailed invitations), October 2025
- Respondents were **primarily employed by health plans** (77%) and represented US **national and regional membership**
- Primary **job function: Formulary/Drug Use Management** (57%)
- Most (53%) were **voting members of the Pharmacy & Therapeutics (P&T) committee**

US Payers Are Not There Yet – They need guidance to understand and integrate PED into decision-making

US Payers and PED: Perspectives Vary (1/2)

- **Most respondents** (87%) said their **understanding of PED completely or mostly aligns FDA's definition**, **however**…
- Some cited **incorrect sources of PED** (e.g., hospitalizations, claims data) or **didn't consider that PED could be valid, reliable, objective**
- **Low level of importance placed on PED as evidence** in formulary decision-making process (53%); **PED** being **used sometimes** (52%)

Many misconceptions, e.g., PED are non-quantifiable, non-reproducible, not available at launch

US Payers and PED: Perspectives Vary (2/2)

- Most (72%) reported considering **whether outcomes assessed in clinical studies matter to patients**
- **Most** (72%) reported their organization being **somewhat open to using PED for formulary decision-making**
- Most agreed **PED has much more influence if available at time of approval** (77%) and as **primary or key secondary endpoint** (90%)

Payers are unclear on **PED's role in drug development** and **how to incorporate PED** into decision-making

US Payers and PED: In their words (1/2)

"**Longstanding policy is to not use patient reported measures** as they are found to be **too subjective**"

"**I don't think as a health plan we are at a place where we would trust any PED**. We are already skeptical enough of the clinical trials, **this seems like it could be even more easily manipulated**"

"**We lack a standard process** and data and ways to evaluate and present to the committees"

US Payers and PED: In their words (2/2)

"...based my new **understanding** of PED... **many drugs are** approved and **placed on our formulary based on a clinical endpoint that is** considered **PED**"

"If **clinical study outcomes ... don't** appear to **have a meaningful connection to the patient, we generally wouldn't value them as much**"

"We **anticipate** that we will **utilize it more frequently to differentiate between agents**"

US Payers and PED: We're Not There Yet

- We need to **overcome skepticism and misconceptions regarding PED, particularly PROs** – *this is foundational to progress in this area*
- We can **partner with payers to develop frameworks for integration of PED** into formulary decision-making – *PED is gaining traction, now is the time to capitalize on this momentum*
- It's not just about delivering PED to payers – we need to **bridge knowledge gaps** and **partner with payers** early in development to **build trust** and align on PED evidence needs; *payers want to learn how to use PED and understand its role in drug development (65% and 55%)*

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NATIONAL PSORIASIS FOUNDATION®

Using Patient Experience Data to Inform Value Assessment for Access Decision Making

Leah M. Howard, JD

President and CEO, National Psoriasis Foundation

Every Psoriatic Disease Journey is Unique

The **National Psoriasis Foundation** is a nearly **60-year-old organization** serving the more than **8 million Americans** living with psoriatic disease.

For those not familiar with psoriasis or psoriatic arthritis (also referred to as psoriatic disease) …

- Chronic, systemic, highly heterogeneous disease
- Impacts: skin, joints, mental health, cardiometabolic comorbidities leading to earlier death and significantly impacted quality of life
- Treatment journey is **non-linear and highly individualized**

There is no "average" psoriasis patient.

Yet value frameworks often assume one.

Patient Experience Data is Foundational *Not Optional*

- Clinical trials ≠ real-world experience
- Clinical trials tell us what ***can*** happen, while patient experience reflects what ***actually*** matters.
- Traditional Value Assessment also tells an incomplete story.
- While focusing on clinical endpoints and cost effectiveness they miss:
  - Variability in patient disease experience and response
  - Treatment burden
  - Quality of life
  - Access barriers

If you don't measure what matters to patients, you won't define value correctly.

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Patient Input in Value Assessment: Using NPF Research to influence Policy

If we don't tell the story of the patient, who will?
If value assessments are going to dictate access, patient perspectives must be considered.

Decades of Real-World Evidence Collection

Structured surveys captured lived experience, treatment burden, and quality-of-life data tell the impacts of psoriatic disease beyond clinical endpoints.

Longitudinal Insights on the Patient Journey

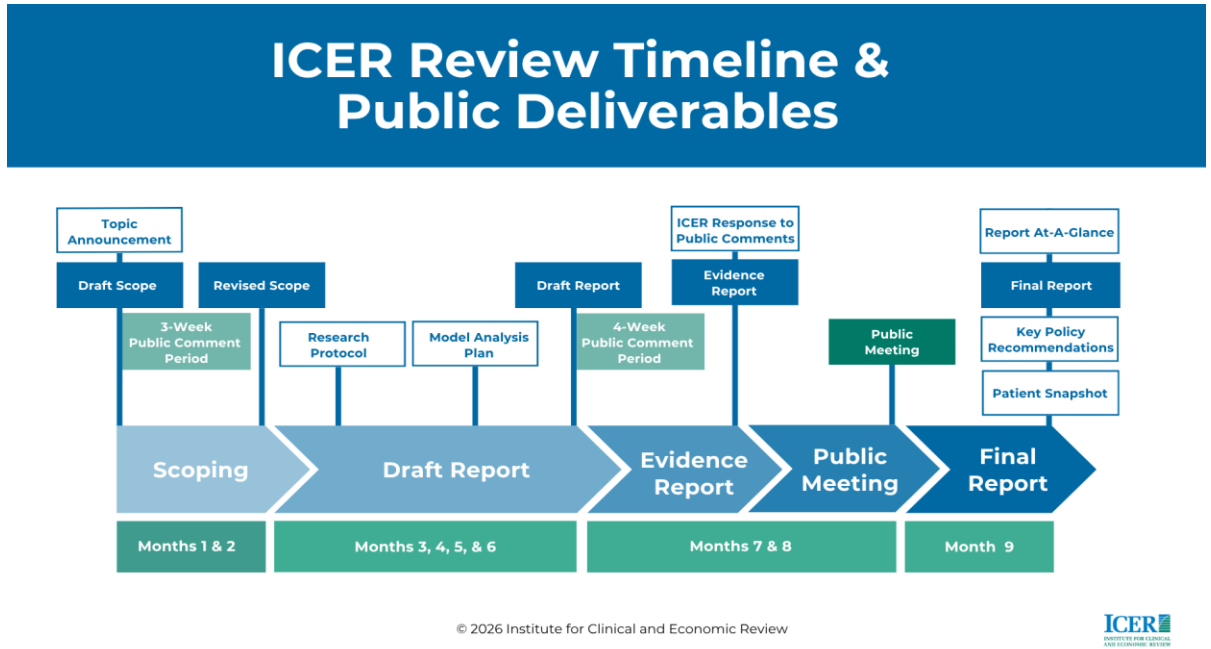
NPF's deep relationships with the psoriatic disease community allowed us to bring real patients from across the community to our work with ICER staff which allowed the sharing of real-life impacts on daily living and mental health.

Deep Relationships with Clinical Experts

Alongside NPF patient data, the NPF Medical Board's three dozen experts in psoriatic disease brought their perspectives complementing patient experiences.

Engagement Strategy and Outcomes: Engage Early, Often, and Strategically

- **Submitted structured evidence during review process**
- **Facilitated direct patient–ICER engagement**
- **Reinforced narratives with clinical and economic context**
  - Multidisciplinary advisory group of patients, medical experts, leadership, and health economists reinforced patient narratives with clinical and economic insights.
- **Engaged at multiple decision points, not just public comment**
  - Goal was to ensure patient perspectives included throughout all stages



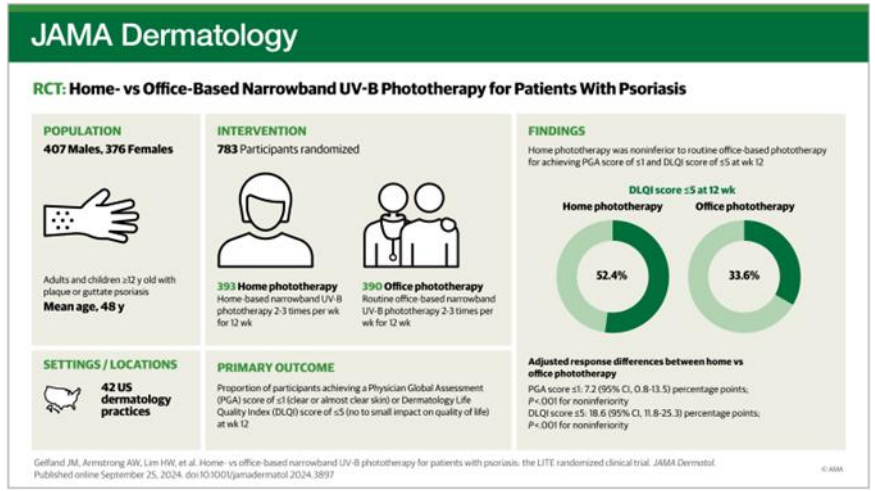
What Moved the Needle?

- Evidence of **treatment burden beyond skin clearance**
- Impact on **mental health and daily functioning**
- Real-world variability in response and adherence
- Barriers created by utilization management
- Need for **treatment sequencing flexibility**

Value assessment should not be only used to limit access.

Beyond ICER: Payer Engagement

- LITE Study: tested the efficacy of home vs. office-based phototherapy for patients across all skin type.
- Designed with **policy and coverage decisions in mind**
- Active payer advisory engagement
- Evidence used to:
  - challenge assumptions
  - inform coverage decisions
  - support access to home phototherapy


JAMA Dermatology

RCT: Home- vs Office-Based Narrowband UV-B Phototherapy for Patients With Psoriasis

POPULATION
407 Males, 376 Females
Adults and children ≥12 y old with plaque or guttate psoriasis
Mean age, 48 y

INTERVENTION
783 Participants randomized
393 Home phototherapy
Home-based narrowband UV-B phototherapy 2-3 times per wk for 12 wk
390 Office phototherapy
Routine office-based narrowband UV-B phototherapy 2-3 times per wk for 12 wk

FINDINGS
Home phototherapy was noninferior to routine office-based phototherapy for achieving PGA score of ≤1 and DLQI score of ≤5 at wk 12
DLQI score ≤5 at 12 wk
Home phototherapy 52.4%
Office phototherapy 33.6%
Adjusted response differences between home vs office phototherapy
PGA score ≤1: 7.2 (95% CI, 0.8-13.5) percentage points; P<.001 for noninferiority
DLQI score ≤5: 18.6 (95% CI, 11.8-25.3) percentage points; P<.001 for noninferiority

SETTINGS / LOCATIONS
42 US dermatology practices

PRIMARY OUTCOME
Proportion of participants achieving a Physician Global Assessment (PGA) score of ≤1 (clear or almost clear skin) or Dermatology Life Quality Index (DLQI) score of ≤5 (no to small impact on quality of life) at wk 12

Gelfand JM, Armstrong AW, Lim HW, et al. Home- vs office-based narrowband UV-B phototherapy for patients with psoriasis: the LITE randomized clinical trial. JAMA Dermatol. Published online September 25, 2024. doi:10.1001/jamadermatol.2024.3897

Consider the end user from the beginning.

Case Study Lessons Learned

- In both cases, **data alongside patient-expressed preferences,** was key
- Design evidence for **policy relevance**
- Engage early and throughout the process
- Pair patient voice with **clinical and economic credibility**

Patient advocacy organizations bring distinct value through deep community insight and patient-centered priorities.

Integrating that expertise into value assessment leads to more informed decisions and better-designed access to care.

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Remarks

Dana McCormick, RPh, FAMCP
Senior Director, Practice Strategy & Innovation
AMCP

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Panel Discussion

Panel Discussion and Q&A

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