Fresh Picks from Blue Persimmon

Insights for Action

This is ***Fresh Picks*** — a new roundup from Blue Persimmon Group.

Each issue will capture the ideas, innovations, and collaborations shaping the future of health, with curated insights that move from idea to impact. Whether it's breakthrough research, system-level innovations, or inspiring collaborations, we'll bring you concise, evidence-driven takeaways to spark action and inform your work.

In this issue, we look at recent meetings where regulators, researchers, and advocates came together to advance patient priorities and accelerate the path to improving outcomes.

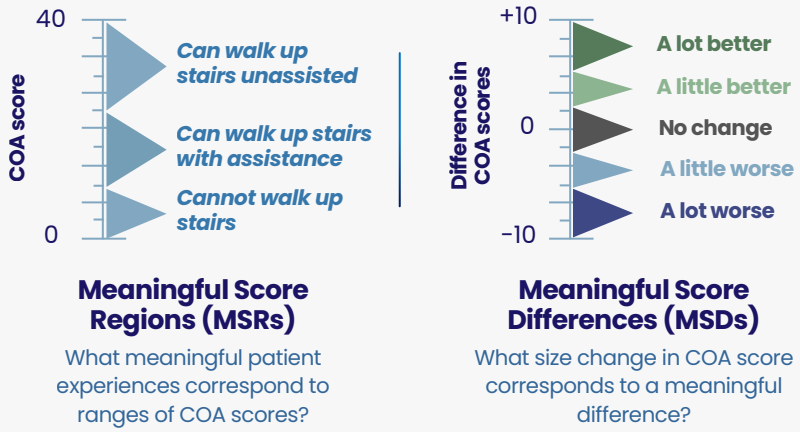
Methodologic and Other Challenges Related to Patient Experience Data

FDA PFDD Workshop – September 18-19, 2025

The FDA hosted a deep dive on elevating patient experience data and clinical outcome assessments (COAs) in drug development. FDA and expert panelists focused on linking COAs to meaningful health outcomes and establishing patient-relevant score ranges to interpret endpoints.

An updated **draft COA Evidence Dossier Template** was introduced to guide sponsor submissions for COA-based endpoints. Case studies were leveraged to: 1) improve clarity regarding the use of the patient voice to interpret COA scores (Figure 1), and 2) elaborate on the importance of measuring meaningful aspects of health to determine treatment effects (Figure 2).

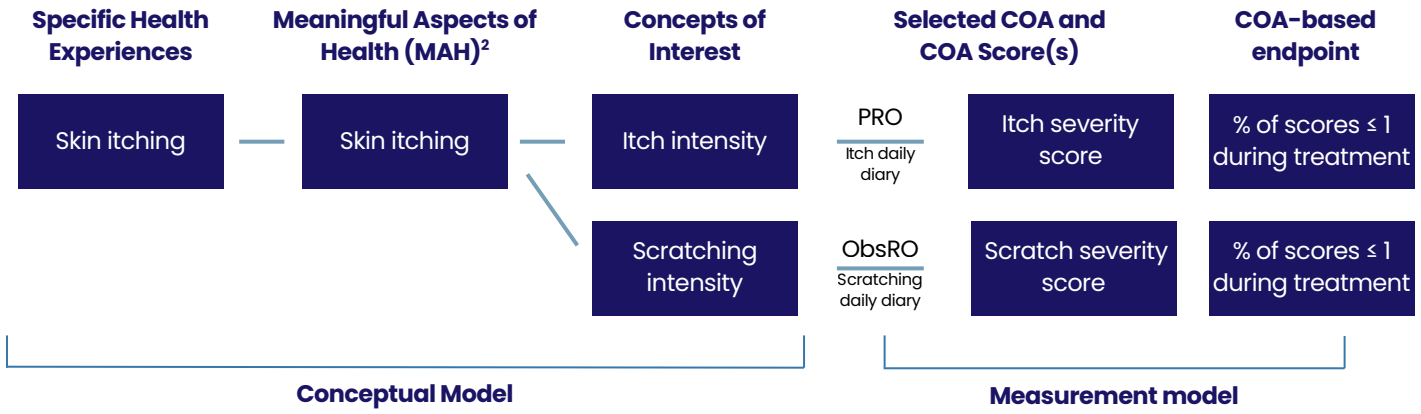
Figure 1. Creating Score Interpretation Methods[1]



Meaningful Score Regions (MSRs)
What meaningful patient experiences correspond to ranges of COA scores?

Meaningful Score Differences (MSDs)
What size change in COA score corresponds to a meaningful difference?

Figure 2. From MAH to Fit-For-Purpose Endpoint - Example Conceptual Framework[1]



[1]Figures adapted from meeting slides and references (**PFDD Draft Guidance 3**, **Edgar 2023**, **PFIC – FDA LEADER 3D Case Study**);
[2]Dimensions of health that are important to patients

PFIC, progressive familial intrahepatic cholestasis; PRO, Patient Reported Outcome; ObsRO, Observer reported outcome

Accelerating Therapies, Modernizing Evidence

Held September 9–11 in Washington, D.C., the 2025 C-Path Global Impact Conference (CGIC) brought together leaders from regulatory agencies, industry, academia, and advocacy to ask: ***how can treatment development move faster, smarter, and in ways that truly work?***

Across three days, discussions spanned **rethinking evidence standards, modernizing trial design, and applying real-world data and digital measures** that meet regulatory scrutiny. Panels on pediatric gene therapy, rare disease study design, and trial innovation showed how **new approaches are narrowing the gap between discovery and approval**.

Panelists stressed that **starting with patient realities speeds trials up**, while regulators highlighted the need for clear endpoints and actionable data. Explore additional highlights by **visiting CGIC 2025**. Meeting recordings will be posted **here.**

FDA Convening: Regulatory Submissions with Real-World Evidence

September 23, 2025, Washington, D.C., Duke-Margolis Institute for Health Policy

- **RWE is being taken seriously by regulators**
  Real-world evidence is actively used in regulatory decisions, supporting labeling for both efficacy & safety
- **Case studies reveal successes and limitations**
  Use cases highlighted RWE's fitness for use and when data quality or bias constrain it
- **Data fitness and transparency are pivotal**
  Credible reviews require well-characterized datasets and transparent, reproducible methods
- **Rigor and pre-specification matter**
  Pre-specified analysis plans, sensitivity checks, and robustness build confidence
- **Collaboration and standardization are key**
  Public-private partnerships, common data standards, and ongoing FDA/sponsor communication improve trust and reproducibility

> "The study leveraged a fit-for-use high quality national registry to provide generalizable findings for the indicated population."

Vishal Bhatnagar, MD
US Food and Drug Administration

From advancing methods for patient experience data to modernizing how real-world evidence informs regulatory decisions and policy, these recent meetings underscore an important advance in person-centered innovation. Embraced by regulators, researchers, and advocates alike, these methods are accelerating treatments for what matters most.

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