

Pediatric Clinical Outcome Assessments: The PROMIS® Approach

ADEPT 6 Workshop, USFDA

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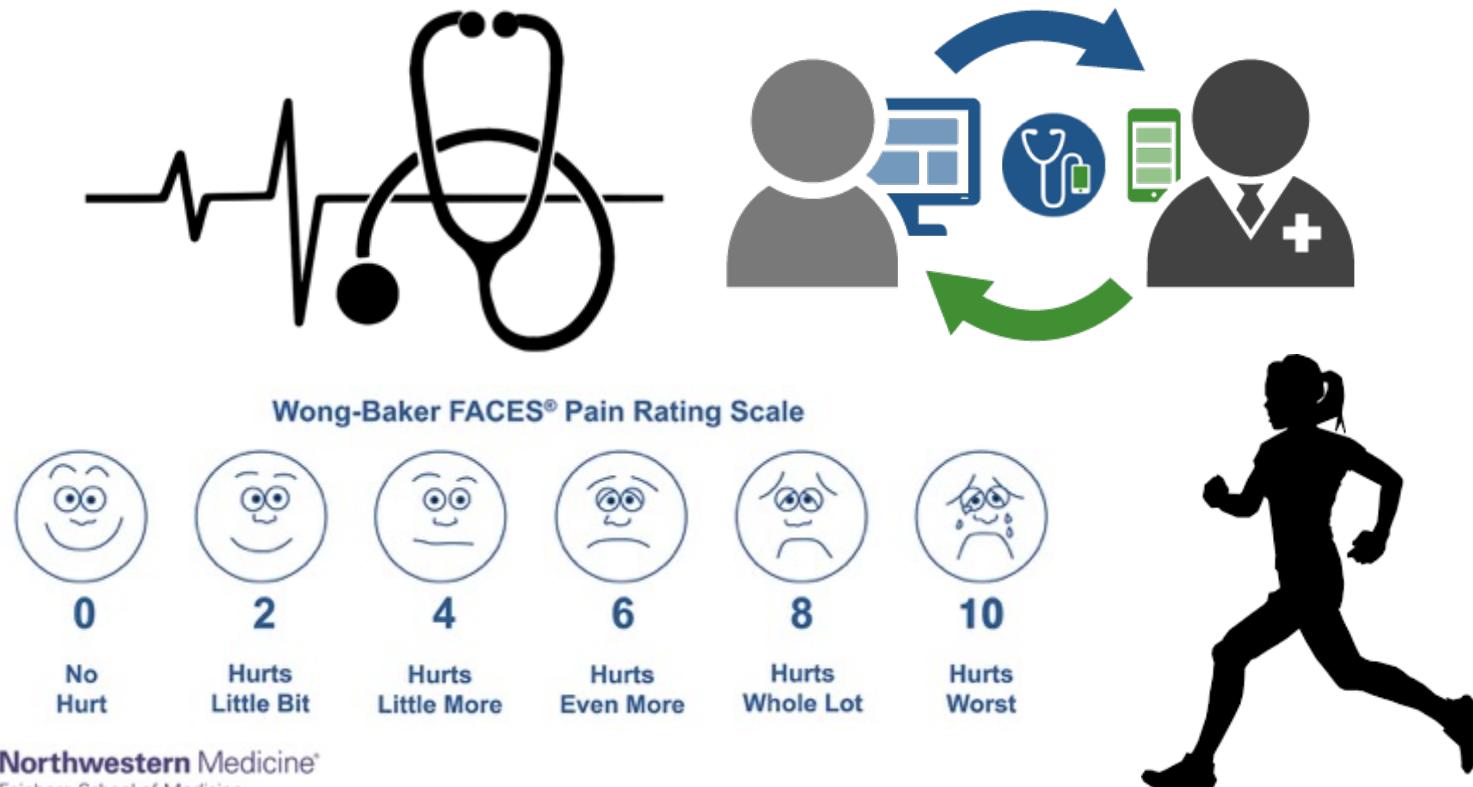
The What, Why, and How of Patient-Reported Outcomes (PROs)

The What: Defining PROs

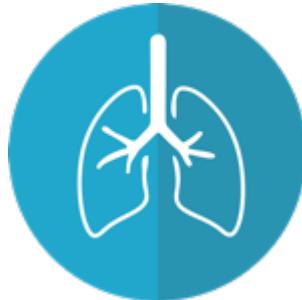
“A measurement based on a report that **comes directly from the patient** (i.e., study subject) about the status of a patient’s health condition **without amendment or interpretation** of the patient’s response by a clinician or anyone else. A PRO can be measured by **self-report or by interview** provided that the interviewer records only the patient’s response.” (USFDA, 2009)



The Why: PROs in Clinical Practice



The How: PRO instruments



The PROMIS® Approach



What is PROMIS®?

Patient-Reported Outcome Measurement Information System®

- Over 300 measures of physical, mental and social health, including >100 for children ages 1-17yrs*

Global Health

Physical Health	Mental Health	Social Health
<ul style="list-style-type: none">- Asthma Impact- Fatigue- Mobility- Pain Intensity- Pain Interference- Pain Behavior- Pain Quality- Physical Activity*- Physical Stress Experiences- Sleep*- Strength Impact- Upper Extremity Function	<ul style="list-style-type: none">- Anger*- Anxiety*- Cognitive Function- Depressive Symptoms*- Engagement**: <i>Curiosity</i> <i>Persistence</i>- Life Satisfaction- Meaning & Purpose- Positive Affect*- Psychological Stress Experiences- Self-Control**: <i>Adaptability</i> <i>Emotion & Behavior Regulation</i>	<ul style="list-style-type: none">- Family Relationships*- Peer Relationships*

What is PROMIS®? (Cont'd)

Patient-Reported Outcome Measurement Information System®

Pediatric Global Health 7+2

Please respond to each question or statement by marking one box per row.

- Short-forms (4- to 10-items), Computer Adaptive Tests (CATs), and profiles
- English and Spanish (and other languages depending on measure)
- Normed to the U.S. general population, validated in clinical subgroups
- Publicly-available on HealthMeasures.net

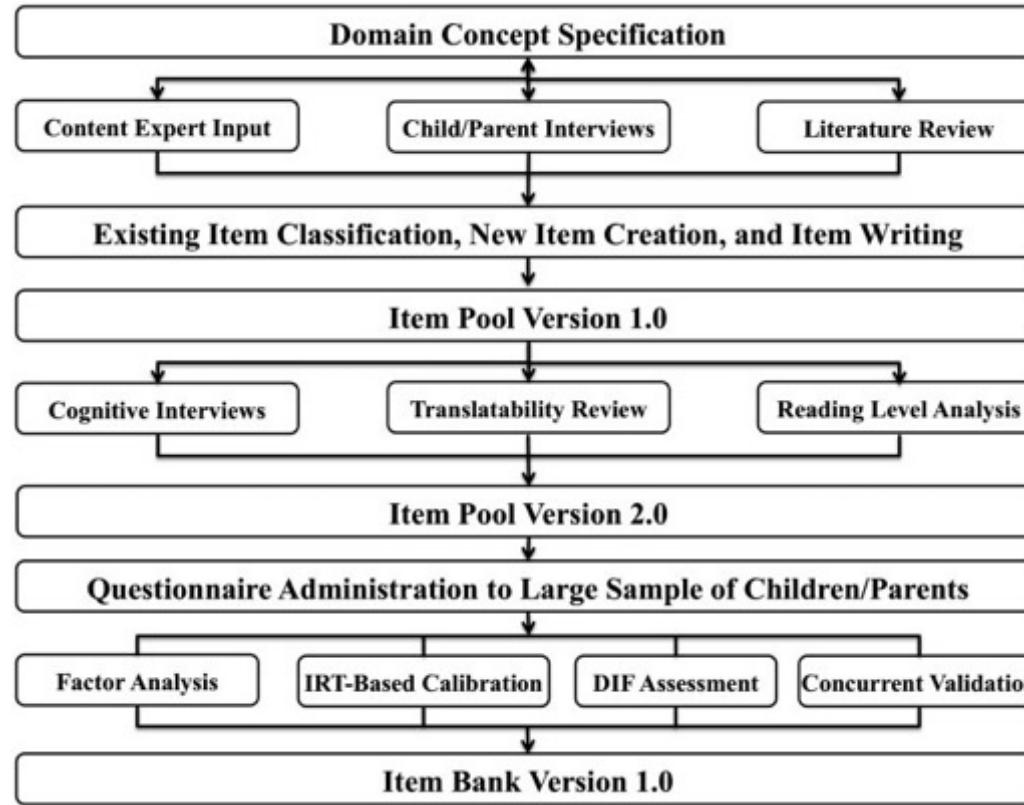
	Excellent	Very Good	Good	Fair	Poor
In general, would you say your health is:.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
In general, would you say your quality of life is:.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
In general, how would you rate your physical health?	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
In general, how would you rate your mental health, including your mood and your ability to think?	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
	Never	Rarely	Sometimes	Often	Always
How often do you feel really sad?	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
	Always	Often	Sometimes	Rarely	Never
How often do you have fun with friends?	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
How often do your parents listen to your ideas?.....	<input type="checkbox"/> 5	<input type="checkbox"/> 4	<input type="checkbox"/> 3	<input type="checkbox"/> 2	<input type="checkbox"/> 1
In the past 7 days...					
	Never	Almost Never	Sometimes	Often	Almost Always
I got tired easily	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5
I had trouble sleeping when I had pain.....	<input type="checkbox"/> 1	<input type="checkbox"/> 2	<input type="checkbox"/> 3	<input type="checkbox"/> 4	<input type="checkbox"/> 5

The PROMIS® Approach

Concept Interviews

Cognitive Interviews

Calibration & Norming

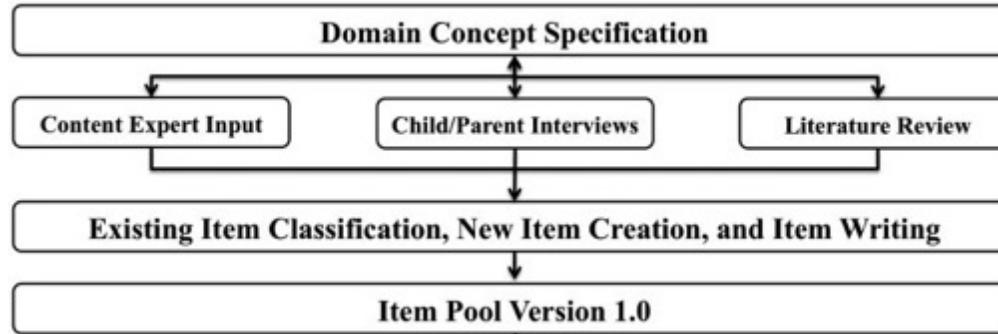


Concept Interviews

Concept Interviews

Cognitive Interviews

Calibration & Norming



- ~10-20+ participants in the target population, depending on saturation
 - Continue interviews until no new information
- Aim for sufficient coverage across child ages
 - e.g., at least 2 parents of 12-18mo, 19-24mo, 25-36mo, 37-48mo, 49-60mo
 - e.g., include children from across the 8-17yr old age range

Concept Elicitation Interview Example

Positive Affect

Respondents	Probe	Response examples
Parents of 1-5yr olds	How would you describe your child when he/she is in a positive mood?	<ul style="list-style-type: none">• Playful, bubbly• Energetic, upbeat• Smiling, laughing, giggly• Happy, jovial, joyful, jubilant• Positive attitude• Loving, cuddly
8-17yr old children	How do kids feel when they have a good life?	<ul style="list-style-type: none">• Happy• Proud• Grateful• Confident, feel good about themselves• Can be what they really want to be• Excited, playful¹

Concept Elicitation Interview Example

Family Relationships

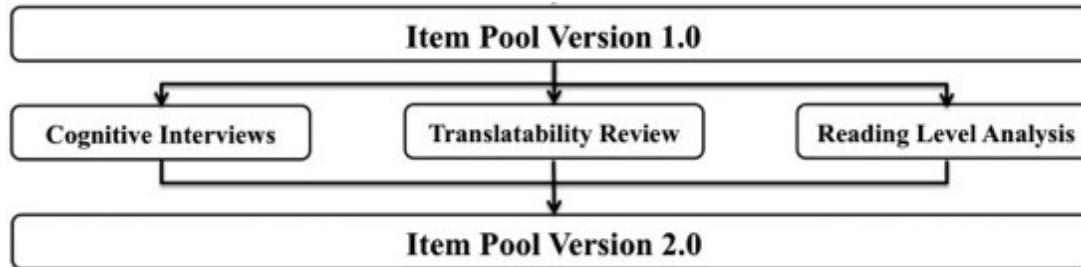
Respondents	Probe	Response examples
Parents of 1-5yr olds	How would you describe a “good quality” relationship between children and caregivers?	<ul style="list-style-type: none">• Loving, telling him I love him• Trust• Caring, nurturing• Being engaged in their lives• Doing activities together, spending a lot of time together
8-17yr old children	What is important about “family” to kids your age?	<ul style="list-style-type: none">• Families love and care• Able to communicate with parents• Accepted for who I am• Lets me do what I want to do• Families stick together¹

Concept Elicitation Interview Example

Sleep Health

Respondents	Probe	Response examples
Parents of 1-5yr olds	What does “poor quality” sleep mean to you in terms of your child?	<ul style="list-style-type: none">• Talking in sleep• Grinding teeth• Sleeping in our bed• Tossing and turning• Waking up through the night• Bad dreams, nightmares, night terrors• Wetting the bed
8-17yr old children	Can you describe instances when you had a “hard” time sleeping?	<ul style="list-style-type: none">• Talking in sleep• Grinding teeth• Snoring, breathing problems• Trouble falling asleep• Can’t stay asleep• Nightmares¹

The PROMIS® Approach



- Every item reviewed by:
 - ≥ 5 participants in the target population
 - Non-white participants
 - Individuals with <HS education (for adults)
- Re-test items with major revisions with ≥ 2 more participants
- Final item sets undergo Lexile analysis to determine reading level
 - Aim for $\leq 6^{\text{th}}$ grade reading level



Cognitive Interview Examples

PROMIS Early Childhood Global Health

Original Item	Feedback	Revision
<i>How would you rate your child's social health?</i>	<p>“I guess the word health kind of throws it off a little bit, when you're thinking of their ability to interact with other kids. That's just kind of how I interpreted the question.” (19-24mo)</p> <p>“I would say something along the lines of social skills maybe rather than health. Don't know if there's a difference between social health and social skill sets so, I answered it as a social skill.” (25-36mo)</p> <p>“I guess the way I understood the question was how he relates to social activities with other kids...I mean I understood what it said, [but] there was a different way to say that one. Maybe social interaction I guess.” (49-60mo)</p>	<p>Retested new item:</p> <p><i>How would you rate your child's social skills?</i></p> <p>All 6 parents in the re-test preferred “social skills” over “social health.”</p>

Cognitive Interview Examples

PROMIS Early Childhood Self-Regulation

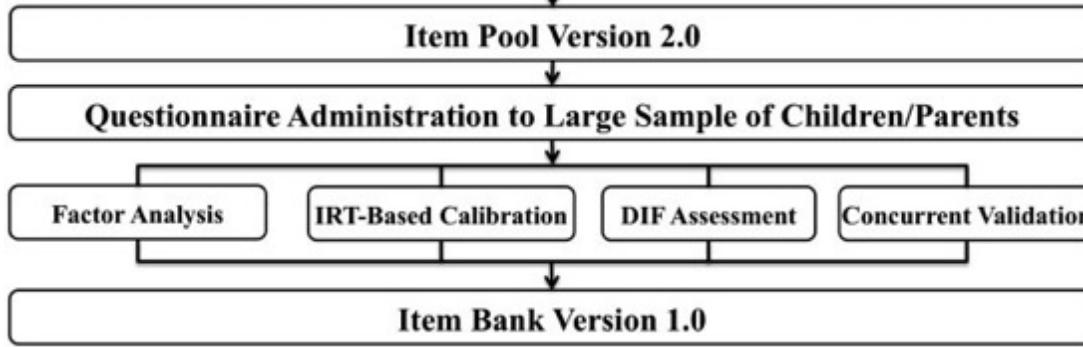
Original Item	Feedback	Revision
<i>It takes a lot for my child to get frustrated.</i>	<p>“A lot of what, right? Like, what is a lot?” (12-18mo)</p> <p>“I’m going to say rarely. Which means that he gets frustrated easily, right? I’m understanding that?” (19-24mo)</p> <p>“It’s a lot of stuff that make her mad.” (37-48mo)</p>	<p>Drop item and maintain similar but clearer item:</p> <p><i>My child managed frustration well.</i></p>

Cognitive Interview Examples

PROMIS Early Childhood Physical Activity

Original Item	Feedback	Revision
<i>How many days did your child move around so much that he/she needed to stop and catch his/her breath?</i>	<p>“Not as [relevant] just because she can’t really get up a lot of speed.” (12-18mo)</p> <p>“My child is very, very, very active. Like he’ll take his little scooter and zip it around the house like 45 times just in circles but he hardly ever gets out of breath. I don’t think he ever does anything to the point where he needs to kind of stop.” (19-24mo)</p> <p>“I don’t see any child at that age doing physical activity so hard that they can’t catch their breath, they have to stop to catch their breath.” (49-60mo)</p>	Drop item because developmentally inappropriate

The PROMIS® Approach



- Large-scale nationally representative survey of target population
- Check people from different demographic and/or clinical subgroups answer the items in the same way
- Further validation with additional clinical subgroups



A Note on Self-Reporting: How Young Is Too Young?



General Guidelines

- If individuals are the most reliable and accurate reporters of their own health, then **gold standard is children reporting for themselves**
- Assumption: Most children as young as 8 have the mental functions needed to understand items and response options, evaluate and summarize their experiences relative to item meaning, and select response options that best represent their self-evaluation
- But every child is different, and **normative variation in cognitive capacities** is especially pronounced among school-aged children
 - Challenge for PRO measurement

Child Self-Report Data Quality Indicators

- Goals:
 - 1) Develop recommendations for self-report capacity screening
 - 2) Use indicators as outcomes in research on test accommodation methods (e.g., audio/video/illustrated items, adapted response scales, etc.)

Child Self-Report Data Quality Indicators

- Archival indicators applied *after* data collection
 - Secondary analyses of 8-11yr olds' responses to Healthy Pathways Child Report Scales
 - Common indicators were not useful but more computationally-intensive approaches are not practical for real-world settings
 - Reverse-coded item inconsistency may be best “quick check” (use with caution)
- Direct indicators applied *during* data collection
 - Attentiveness: eye-tracking
 - Understanding + response logic: intensive cognitive interview
 - Reading comprehension

References

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Thank You

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