Person-Centered Outcome Instruments:
PROMIS, Neuro-QOL, and NIH Toolbox

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Patient-Reported Outcomes (PROs)

- Quantification of symptoms, functional status, perceptions of health
- Subjective and multi-dimensional
Why ask patients?

• Best source of information
  – Symptoms or health-related quality of life (HRQL): pain, fatigue, distress
  – Impact of symptoms on continuing meaningful activities
  – Knowledge, attitudes, behavior
  – Satisfaction
• Same biological value in 2 pts ≠ same impact
• Variability in correlation b/w clinician and pt reports
• HRQL scores (esp physical fx) predicts survival in many conditions\(^1\)

Carrots and Sticks for PROs

• Era of patient-centeredness

Meaningful Use: Core Objectives

• 3rd level health IT meaningful use incentive program
  – CMS gathering info on PRO-based performance measures

• PCORI
Problems with PRO Measures

• Ceilings and floors

*Being John Malkovich*

1999
Measurement Ceiling

- PROMIS Anger CAT (4-12 items, median=5-6)
- Aggression Questionnaire (12 items)

Effect Size

Baseline | 1-month | 3-month
--- | --- | ---
0 | 0.40 | 0.40

Time Point

Problems with PRO Measures

- Ceilings and floors
- Patient burden
- Royalties/costs
- Psychometric limitations
- Scores w/o interpretation guidelines

*Being John Malkovich*
1999
It is impractical to use disease-/condition-specific instruments
New Measurement Initiatives

• Patient-Reported Outcomes Measurement Information System (PROMIS)

• Quality of Life in Neurological Disorders (Neuro-QOL)

• NIH Toolbox for Assessment of Neurological and Behavioral Function (NIH Toolbox)
Approach to Measurement

• Non-disease/condition specific (universal)
• Domains of self-reported health
  – NIH Toolbox – also performance measures
• Item Response Theory
• State of the science mixed methods

Desired Aims
• More widely applicable
• More sensitive
• More efficient
PROMIS

• Item banks – self report physical, mental, social health
• Use across chronic conditions
• Ages 8 – adult (self report). Age 5 proxy.
• CAT, short forms
• Link scores between measures

www.nihpromis.org
PROMIS Domain Framework

Self-Reported Health

Physical Health
- Symptoms
- Function

Mental Health
- Affect
- Behavior
- Cognition

Social Health
- Relationships
- Function
PROMIS Physical Health Item Banks

Physical Health

**Adult**
- Pain Behavior
- Pain Interference
- Fatigue
- Sleep Disturbance
- Sleep-related Impairment
- Physical Function
- Sexual Function
- Pain Intensity

**Pediatric**
- Pain Interference
- Fatigue
- Upper Extremity
- Mobility
- Asthma Impact

*Also in Spanish & other languages*
PROMIS Mental Health Item Banks

Mental Health

**Adult**
- Anxiety
- Depression
- Anger
- Psychosocial Illness Impact
- Cognitive Function
- Alcohol Use, Consequences, Expectancies

**Pediatric**
- Anxiety
- Depression
- Anger

*Also in Spanish & other languages*
PROMIS Social Health Item Banks

**Social Health**

**Adult**
- Ability to Participate in Roles & Activities
- Satisfaction with Roles & Activities
- Companionship
- Emotional Support
- Informational Support
- Instrumental Support
- Social Isolation

**Pediatric**
- Peer Relationships

*Also in Spanish & other languages*
In Progress

• Adult
  – GI Symptoms
  – Self-efficacy for management of chronic disease
  – v2.0 Sexual Function

• Pediatric
  – Pain Behavior, Quality, Intensity
  – Physical Activity
  – Experience of Stress
  – Subjective Well-being
  – Impact of Child Illness on Family
  – Family Belongingness
  – Global Health
PROMIS Pain Interference Short Form

In the past 7 days...

<table>
<thead>
<tr>
<th>Question</th>
<th>Not at all</th>
<th>A little bit</th>
<th>Somewhat</th>
<th>Quite a bit</th>
<th>Very much</th>
</tr>
</thead>
<tbody>
<tr>
<td>How much did pain interfere with your day to day activities?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>How much did pain interfere with work around the home?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>How much did pain interfere with your ability to participate in social activities?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>How much did pain interfere with your household chores?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>How much did pain interfere with the things you usually do for fun?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
<tr>
<td>How much did pain interfere with your enjoyment of social activities?</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>

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PROMIS T-score

• Mean = 50
  – Standard Deviation = 10

• Referenced to the US General Population

• High scores = more of domain

• MIDs = 4-6 points (physical function)¹

¹Yost et al 2011
Neuro-QOL

• Item banks – self report physical, mental, social health
• Supplemental domains specific to targeted diseases (e.g., stigma)
• Use in chronic neurologic conditions
• CAT, short forms
• Link scores

www.neuroqol.org
Neuro-QOL Domain Framework

Self-Reported Health

Physical Health
  - Symptoms
  - Function

Mental Health
  - Emotional Health
  - Cognitive Health

Social Health

*Also in Spanish*
Neuro-QOL Physical Health Banks

Adult
- Fatigue
- Sleep Disturbance
- Upper Extremity
- Mobility

Pediatric
- Fatigue
- Pain
- Upper Extremity
- Mobility

*Also in Spanish
Neuro-QOL Mental Health Banks

Mental Health

**Adult**
- Anxiety
- Depression
- Pos Affect & Well-being
- Emotional & Beh Dyscontrol
- Stigma
- Cognitive Function
- Communication

**Pediatric**
- Anxiety
- Depression
- Anger
- Stigma
- Cognitive Function

*Also in Spanish*
Neuro-QOL Social Health Banks

**Adult**
- Ability to Participate in Roles & Activities
- Satisfaction with Roles & Activities

**Pediatric**
- Interactions w Peers

*Also in Spanish*
Neuro-QOL T-score

• Mean = 50
  – Standard Deviation = 10

• Referenced to the US General Population or Clinical Population

• High scores = more of domain
NIH Toolbox

• Assessment of neurological and behavioral function
• Use across diverse populations
• Ages 3-85
• Batteries (CATs, short forms)
  – Cognition, Motor, Sensation, Emotion
• Objective tests and survey measures
• English and Spanish

www.nihtoolbox.org
Cognition Domains

- Executive Function
- Attention
- Episodic Memory
- Language
- Working Memory
- Processing Speed
Motor Domains

- Dexterity
- Strength
- Balance
- Locomotion
- Endurance
Sensation Domains
Emotion Domains

Emotion

Psychological Well-Being
- Positive Affect
- Life Satisfaction
- Meaning & Purpose

Social Relationships
- Social Support
- Companionship

Stress & Self-Efficacy
- Perceived Stress
- Self-Efficacy

Negative Affect
- Fear
- Sadness
- Anger

Positive Social Development

NIH TOOLBOX
MOTOR EMOTION SENSATION COGNITION
NIH Toolbox Scores

- PRO and Performance Measures
  - Mean = 100 (SD=15)
  - Unadjusted Scale Score
  - Age-adjusted scale score
  - Fully adjusted scale score
- PRO “Raw” scores also available to match PROMIS
  - T-score mean = 50 (SD=10)
Advantages to Newer Instruments

• Improved measurement
  – Increased power ➔ reduced sample size
• Adaptability
• Low patient burden
• Comparability
• Royalty-free
Physical Function
Measurement Precision and Range

SF-36 10 items
HAQ 20 items
CAT 10 items
PROMIS Short Form 10 items
PROMIS Short Form 20 items

Error

Fries et al 2009

SE = 3.3
rel = 0.90

SE = 2.3
rel = 0.95

rheumatoid arthritis patients
US general population

5.0 4.0 3.0 2.0 1.0 0.0

10 20 30 40 50 60 70
The shaded areas represent the effective measurement ranges where a reliability of roughly .95 is attainable.
Measures of Responsiveness

- **Effect size (ES)** = \( \frac{\text{Mean change}}{\text{SD at baseline}} \)

- **Standardized response mean (SRM)** = \( \frac{\text{Mean change}}{\text{SD of change for the sample}} \)

- **Responsiveness statistic (RS)** = \( \frac{\text{Mean change}}{\text{SD of change for group who did not change}} \)
Physical Function Responsiveness – Effect Sizes

- Better (N=75): 0.29 (PROMIS), 0.28 (SF-36)
- Same (N=114): 0.04 (PROMIS), 0.04 (SF-36)
- Worse (N=55): -0.51 (PROMIS), -0.4 (SF-36)

Yost et al. 2011
Advantages to Newer Instruments

• Improved measurement
  – Increased power \(\rightarrow\) reduced sample size
• Adaptability
• Low patient burden
• Comparability
• Royalty-free
### Adaptability

#### Patients: Items

<table>
<thead>
<tr>
<th>Patients</th>
<th>Low PF</th>
<th>New Item</th>
<th>New Item</th>
<th>New Item</th>
<th>New Item</th>
<th>New Item</th>
<th>High PF</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>PC03</td>
<td>PC04</td>
<td>PC05</td>
<td>PC06</td>
<td>PC07</td>
<td>PC08</td>
<td></td>
</tr>
<tr>
<td>2</td>
<td>PC11</td>
<td>PC12</td>
<td>PC13</td>
<td>PC14</td>
<td>PC15</td>
<td>PC16</td>
<td></td>
</tr>
<tr>
<td>3</td>
<td>PC21</td>
<td>PC22</td>
<td>PC23</td>
<td>PC24</td>
<td>PC25</td>
<td>PC26</td>
<td></td>
</tr>
<tr>
<td>4</td>
<td>PC31</td>
<td>PC32</td>
<td>PC33</td>
<td>PC34</td>
<td>PC35</td>
<td>PC36</td>
<td></td>
</tr>
<tr>
<td>5</td>
<td>PC41</td>
<td>PC42</td>
<td>PC43</td>
<td>PC44</td>
<td>PC45</td>
<td>PC46</td>
<td></td>
</tr>
</tbody>
</table>

*Hung et al. 2011*
Advantages to Newer Instruments

• Improved measurement
  – Increased power \(\rightarrow\) reduced sample size
• Adaptability
• Low patient burden
• Comparability
• Royalty-free
Fast Completion Times

- 66 seconds
  - Lower Extremity CAT
  - Patients scheduled for foot and ankle surgery\(^1\)
- 98 seconds
  - Physical Function CAT
  - Proximal humerus fractures in patients > 60 years old\(^2\)

\(^1\)Hung et al 2012
\(^2\)Vrahass under review
Advantages to Newer Instruments

• Improved measurement
  – Increased power → reduced sample size

• Adaptability

• Low patient burden

• Comparability

• Royalty-free
PROMIS Fatigue Across Five Clinical Conditions

- **Cancer**
  - N = 310

- **Back Pain**
  - N = 229

- **Depression**
  - N = 114

- **Heart Failure**
  - N = 64

- **COPD**
  - N = 125

Average for General Population

- **Back Pain**
  - Stable (B)
  - Exacerbation (B)
  - (1 mo)
  - (3 mos)

- **Depression**
  - (B)
  - (1 mo)
  - (3 mos)

- **Cancer**
  - Chemo (B)
  - (2 mos)

- **Heart Failure**
  - Pre-transplant
  - Post-transplant

- **COPD**
  - Stable (B)
  - Exacerbation (B)
PROsetta Stone

- Linking tables
  - Legacy instruments to PROMIS metric
- BPI, CES-D, FACIT, HAQ, SF-36, PHQ-9, etc
- www.prosettaSTONE.org
Advantages to Newer Instruments

• Improved measurement
  – Increased power ➔ reduced sample size
• Adaptability
• Low patient burden
• Comparability
• Royalty-free
How Do I Choose a Measure?

• Population
• Age
• Domains
  – See definitions, review items
  – PROMIS Scoring Manuals
• Aims of measurement (e.g., screening, primary outcome)
  – Precision
• Completion time
To Learn More

- www.nihpromis.org
- www.neuroqol.org
- www.nihtoolbox.org
- Video tutorials
- Publications
- Manuals
Acknowledgements

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Thank you!