PROMIS & ASCQ-Me vs. Older SCD-Specific Measures: the SHIP-HU Study

Wally R. Smith, MD, Donna K. McClish PhD, Richard Lottenberg, MD, Ian Chen, MD, Marsha Treadwell, PhD, India Sisler, MD, Shirley Johnson, BA, Daniel Sop BS

NHLBI 1R18HL112737

NCT02197845
Sickle Cell Disease

- Worldwide Genetic hemoglobinopathies
- 100,000 in US, predominately African, Mediterranean descent
- Extreme, unpredictable acute → chronic pain
- Vaso-occlusive ischemia, organ failure, early death
- Few treatments
SCD: A Double Whammy

- Members of a Health Disparities population
- Suffer Chronic Non-Cancer Pain (CNCP)
  - CNCP Common
  - Significant burden to adult patients
  - Known to lower quality of life
- May be poorly managed
  - → anxiety and distress related to subsequent treatment
  - → negative long-term psychological effects
Healthy children

African Americans in urban Milwaukee, WI

Cancer: off treatment

African Americans in Cincinnati, OH

Sickle cell disease

Cancer: on treatment

Severe obesity

Cystic fibrosis

Cancer

Asthma

Sickle cell disease

Healthy adults

Patients on dialysis

Healthy paediatric population

Healthy adult population

Children with illness

Adults with illness
## Places Where PROs Might be Needed in Sickle Cell Disease

<table>
<thead>
<tr>
<th>Disease Modifying</th>
<th>Palliative (analgesic)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acute</td>
<td>Acute</td>
</tr>
<tr>
<td>Chronic</td>
<td>Chronic</td>
</tr>
<tr>
<td>Organ preserv</td>
<td></td>
</tr>
</tbody>
</table>


Adult Sickle Cell Quality of Life Measurement (ASCQ-Me)

Describes the broader effect of SCD on adult functioning and well being to:

- More fully characterize the efficacy of therapies tested in RCTs
- Evaluate the effectiveness of therapy
- Compare effectiveness of therapies
- Evaluate effectiveness of healthcare delivery system
ASCQ-Me/PROMIS Conceptual Model

Interventions
- Medical care
- Coping techniques

Pain
- Impact
  - Very Severe
  - Urgent/unpredictable

Emotional Distress
- Anxiety
- Depression
- Anxiety, depression about Health

Physical Distress
- Fatigue
- ADL/IADL Impact
- Stiffness

Role Interference
- Social
- Family
- Work (paid/unpaid)

Key:
- ASCQ-Me
- PROMIS

Treadwell et al *Clin J Pain* 2014;30;902-14
Keller et al *Health Qual Life Outcomes* 2014;12:125
Objectives

- Test the relationship between function, barriers to care in physical function domain versus those in psychosocial domain, medical skill set domain.
- Compare scores from SCD PROs previously measured and felt important vs. PROs in SCD.
  - Patient Reported Outcomes Measurement Information System, PROMIS
  - Adult Sickle Cell Quality of Life Measurement Information System, ASCQ-Me
- Compare physical function scale scores from PROMIS and ASCQ-Me in a SCD sample.
Patients

- N=214, 53% female
- Enrolled in Start Healing in Patients with Hydroxyurea (SHIP-HU) Clinical Trial
  - NHLBI 1R18HL112737
  - PI Wally R. Smith, MD
- 79% prescribed HU at baseline
Measures

- **PROMIS**
  - Global health – physical summary (GPHYS)
  - Physical health (PHYS)
  - Fatigue (FATG)
  - Pain behavior (PAINB)

- **ASCQ-Me**
  - Sleep/Wake disturbance (WAKE)
  - Stiffness (STIFF)
  - Sleep impact (SLEP)
  - Pain crisis frequency (PAINF)
  - Pain crisis severity (PAINS)
  - Pain impairment (PAINI)

- Smith-Bovbjerg Sickle Cell Stress scale (STRESS)
- Social support (SS)
- Sickle Cell Self efficacy (SCSES)
- Medical Knowledge and Skills (MEDSKILL) scales, Transition Intervention Program - Readiness for Transition
- Three subscales of Coping Strategies Questionnaire (Emotion-focused, Active, Behavioral-- COPEEMOT, COPEACT, COPEBEH)
Results--Correlations

<table>
<thead>
<tr>
<th></th>
<th>GPHYS</th>
<th>PHYS</th>
<th>FATG</th>
<th>WAKE</th>
<th>STIFF</th>
<th>SLEP</th>
<th>PAINB</th>
<th>PAINF</th>
<th>PAINS</th>
<th>PAINI</th>
</tr>
</thead>
<tbody>
<tr>
<td>STRESS</td>
<td>-0.44***</td>
<td>-0.36***</td>
<td>0.32**</td>
<td>0.34***</td>
<td>-0.37*</td>
<td>-0.25*</td>
<td>0.25*</td>
<td>0.31</td>
<td>0.21*</td>
<td>-0.46***</td>
</tr>
<tr>
<td>SS</td>
<td>0.04</td>
<td>0.14</td>
<td>-0.08</td>
<td>-0.11</td>
<td>0.04</td>
<td>0.09</td>
<td>-0.12</td>
<td>-0.1</td>
<td>0.01</td>
<td>0.12</td>
</tr>
<tr>
<td>SCSES</td>
<td>0.29**</td>
<td>0.21*</td>
<td>-0.28*</td>
<td>-0.25*</td>
<td>0.12</td>
<td>0.22*</td>
<td>-0.2</td>
<td>-0.23*</td>
<td>-0.23*</td>
<td>0.27*</td>
</tr>
<tr>
<td>MEDSKILL</td>
<td>-0.09</td>
<td>-0.05</td>
<td>-0.03</td>
<td>-0.07</td>
<td>-0.1</td>
<td>-0.02</td>
<td>-0.05</td>
<td>-0.08</td>
<td>0</td>
<td>0.02</td>
</tr>
<tr>
<td>COPEACT</td>
<td>-0.12</td>
<td>-0.01</td>
<td>0.12</td>
<td>0.11</td>
<td>-0.18*</td>
<td>-0.15</td>
<td>0.11</td>
<td>-0.04</td>
<td>0.1</td>
<td>-0.03</td>
</tr>
<tr>
<td>COPEEMO</td>
<td>-0.33**</td>
<td>-0.23*</td>
<td>0.35***</td>
<td>0.44***</td>
<td>-0.37***</td>
<td>-0.25*</td>
<td>0.25*</td>
<td>0.12</td>
<td>0.02</td>
<td>-0.26</td>
</tr>
<tr>
<td>COPEBEH</td>
<td>-0.05</td>
<td>0.04</td>
<td>0.13</td>
<td>0.08</td>
<td>-0.1</td>
<td>-0.12</td>
<td>-0.04</td>
<td>0.03</td>
<td>-0.02</td>
<td>0.08</td>
</tr>
</tbody>
</table>
Results

- The strongest correlations of physical domain PROs were with STRESS, SCSES, and COPEEMOT.
- Physical domain PROs did not correlate with MEDSKILL, COPEBEH, or (usually) COPEACT.
- Psychosocial domain scores from ASCQ-Me and PROMIS strongly correlated with many scores from older psychosocial measures:
  - STRESS
  - SS
  - SCSES
  - Emotion-focused Coping
- Psychosocial domain scores from ASCQ-Me and PROMIS did not correlate with Medical Knowledge/Skills, Active Coping, or Behavioral Coping.
Conclusions

- In SHIP-HU, strong relationships were found between ASCQ-Me and PROMIS psychosocial PROs when they were compared to previously validated and reported psychosocial PROs.

- Relationships were also found between physical domain PROs and stress, self-efficacy, and (only) emotion-focused coping.

- Along with these relationships, the relative brevity of the newer measures and the ability to compare PROMIS scores to national norms lends credence to their utility for future SCD research.