

Quality of Life in Juvenile Myositis



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Disclosures



- **No relevant disclosures**

Measuring Quality of Life?



- **Patient-Reported Outcomes (PROs)**
 - **Goal:** Determine what outcomes are **most important to patients/families**
 - Find ways to rigorously measure these outcomes
 - Patient (or parent) report should be central to the approach

Why Does PRO Research Matter?



- **Helps us study questions that truly matter to the children and families we serve**
- **Patient/family perspective may differ importantly from physician/researcher perspective**
- **Patient and parent/family perspectives may differ**

Why Does PRO Research Matter?



- **Some outcomes are very important but subjective**
- **Asking people directly about subjective topics is most accurate approach:**
 - Quality of life
 - Mood
 - Participation in daily activities
- **PROs allow us to study how treatments, physical therapy, etc affect these areas too**

Developing Patient-Reported Outcomes?



- Often starts with **qualitative** research
- Developing large sets of questions (“item banks”)
 - “winnowing” / “binning”
- Cognitive interviewing – do the questions make sense to readers?

Developing Patient-Reported Outcomes?



- Usually leads to development of a survey
- **Psychometric** study of the survey
 - Assess if it is measuring what you think it is measuring (**Validity**)
 - Assess if the measurements are similar when different people rate at different times (**Reliability**)

Measuring Quality of Life



- **Existing PROs that have been used to measure quality of life in juvenile myositis include:**
 - Childhood Health Assessment Questionnaire (CHAQ)
 - Child Health Questionnaire (CHQ-PF50)
 - PedsQL (Generic Core Scales and Rheumatology Module)
- **Physical function measured by all of the above**
 - CHQ-PF50 and PedsQL also assess psychosocial quality of life, including social roles (school)

PROMIS – Role in Juvenile Myositis?



- **NIH-funded project → improve PRO measurement for all adult/pediatric populations**
 - Based at Northwestern University
- **Item banks currently available:**
 - Physical Function (Upper Extremity, Mobility), Pain, Fatigue, Depression, Anxiety
 - More item banks being developed/validated currently
- **CureJM-funded study to validate PROMIS in juvenile myositis**
 - Already being used in juvenile idiopathic arthritis, has been validated in pediatric lupus

Benefits of PROMIS



- **Computerized adaptive testing = gold standard**
 - Selecting questions algorithmically from a larger item bank
 - Also available as fixed short forms (paper)
 - Item response theory
 - Differential item functioning, etc
- **More precise measurement = helps study rare diseases**
- **May overcome ceiling/floor effects = makes clinical change easier to demonstrate**
- **Lower respondent burden**
- **Can directly compare pediatric/adult scores**
- **Can compare to other pediatric disease populations**
- **Free to use**

So...What is Known Re: Quality of Life in JDM?



- Lower physical (and possibly psychosocial) QoL than kids who do not have JDM
 - ~10-13% with some significant decrease in QoL
 - Lower physical may persist into adulthood
 - **BUT QoL has been improving each decade**
- Associated with lower QoL
 - Disease activity, muscle weakness
 - Chronic continuous course
 - Calcinosis
 - Pain
 - Fatigue
 - Sleep disturbance

Unanswered Questions on QoL/PROs in JM



- **Most studies purely descriptive**
 - Not much knowledge of how to directly improve QoL?
- **Incomplete information for risk stratification**
- **Limited understanding of how skin disease may affect QoL**
- **Burdens of treatment on QoL?**

Unanswered Questions on QoL/PROs in JM



- Effects of physical therapy, physical activity, and sedentariness on QoL?
- Effects on caregivers and siblings?
- **Limited knowledge of mental health needs in JM**

Conceptual Issues in QoL: Strengths vs Deficits



- **Most traditional QoL/PRO surveys use a deficit-based approach**
 - Assumption #1: Perfect health = best possible QoL
 - Assumption #2: Worsening health = worsening QoL
 - Oversimplification?
- **Role of coping?**
- **How to measure strengths and deficits?**
 - Identifying thriving or resilient outcomes, rather than just the absence of problems