REiNS Patient Reported Outcomes Working Group

REiNS Committee Meeting 6/13/17

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On behalf of the REINS PRO group

Response Evaluation In Neurofibromatosis & Schwannomatosis
INTERNATIONAL COLLABORATION

REiNS PRO Working Group

Taryn Allen, PhD	National Cancer Institute
Andrea Baldwin, CRNP	National Cancer Institute
Amanda Bergner, MS CGC	Johns Hopkins University
Kim Bischoff	NF Network
William Dudley, PhD	UNC-Greensboro
Rosalie Ferner, MD	Guy's Hospital
Barbara Franklin, BS	Patient Advocate
Chris Funes, MS	MGH/Harvard
Kathy Gardner, MD	University of Pittsburgh
Deborah Gold, MD	University Hospitals of Cleveland
Cynthia Hingtgen, MD, PhD	Indiana University
Staci Martin, PhD	National Cancer Institute
Vanessa Merker, BS	MGH/Harvard
Betty Schorry, MD	Cincinnati Children's Hospital
Heather Thompson, PhD. CCC-SLP	California State University
Jim Tonsgard, MD	University of Chicago
Ana-Maria Vranceanu, PhD	MGH/ Harvard
Karin Walsh, PsyD	Children’s National Medical Center
Pam Wolters, PhD	National Cancer Institute

Patient Reported Outcome (PRO)

- "…any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else"
- One type of clinical outcome assessment (COA) used:
  - Assess patient's *subjective perception* of the severity of a specific symptom or disease.
  - Assess patient's *subjective perception* of the impact of disease or treatment.

PRO Working Group

- Goal: To identify PRO measures appropriate for assessing *subjective* clinical benefit in NF trials
- Context of Use:
  - Treatment trials for tumors: FDA is requiring a reduction in tumor volume *in conjunction* with demonstrated patient reported clinical benefit.
    - PRO as a co-primary or secondary endpoint.
  - Psychosocial interventions for symptoms or quality of life: Studies need to show patient reported clinical benefit
    - PRO as a primary endpoint

Core PRO Domains

(≥8 years old)

1) Pain
   - Pain intensity
   - Pain interference
2) Physical Functioning
   - Mobility
   - Upper extremity
3) Disease Specific QOL
   - NF1
   - NF2
   - Schwannomatosis
4) General QoL

Current Work of the PRO Group

Core PRO Endpoint Domains (≥8 years)

- General QOL
- Subgroups for specialized domains:
  - Pain assessment in young children (< 8 years)
  - Hearing and communication

General QoL

- Challenges:

  -There are hundreds of measures available.

  -Difficult to find measures that go from childhood to adulthood.

  - Lack of consensus regarding the need for a general health QoL measure for NF.

General QoL

PROS

- Main outcome for psychosocial clinical trials.
  - Goal is improved satisfaction with symptom, ability to function well in spite of symptom (e.g., hearing).
  - Some interventions target QoL improvement regardless of NF type.
- Allows comparison with other populations.

CONS

- May not be necessary for drug clinical trials. Use as secondary outcome for drug trials.
  - Goal is specific improvement in a symptom (e.g., hearing) which is better captured by a disease specific or symptom specific scale.
- Time intensive process/patient burden.

General QoL

- **Methodology for PRO ratings:**
- Identify the most widely used general QoL measures.
- Conduct full group review of these main measures.
- Screen other QoL measures via individual mini-reviews.
- Ensure limited overlap between disease specific and general QoL measures.

General QoL

- **Current measures reviewed:**
- SF-36- full review. (J. E. Ware & Sherbourne, 1992)
- FACT-G- full review. (Cella et al., 1993)
- WHO QoL BREF- full review. (Skevington, Lotfy, & O'Connell, 2004)
- EURO-QOL – full review (The EuroQol Group, 1990)
- SF-12- mini review. (J. Ware, Kosinski, & Keller, 1996)
- PROMIS global health – mini review. (Hays, Bjorner, Revicki, Spritzer, & Cella, 2009)
- PedsQL- mini review. (Varni, Seid, & Rode, 1999)

General QoL

Criteria	SF-36	FACT-G	QoL BREF	Euro-QoL	PROMIS	SF-12	PedsQL
Patient Characteristics	2.25	1.75	2	2.67	3	2	2.75
Published Studies	3	2.5	2.25	2.67	2.75	2.5	2.75
Item/Domain Content	2.5	2.25	2	1.96	2.5	2.5	2.5
Scores Available	2.75	3	2.4	--	2.75	2.5	2.5
Psychometric Data	2.75	2.75	2.5	2.25	3	2.5	2.5
Feasibility	3	3	2.88	2.75	3	2.75	2.5
MEAN (4)	**2.63**	**2.44**	**2.35**	**2.46**	**2.88**	**2.44**	**2.63**

Pain Assessment in Young Children Subgroup

- Taryn Allen, Andrea Baldwin, & Pam Wolters
- Objective: To identify PROs to assess pain in young children < 8 years
- Considerable limitations to existing tools
  - Many measures are too complex, poor psychometric properties or incomplete psychometric data for this age range
  - Many measures are better indicated for *acute* rather than *chronic* pain

Pain Assessment in Young Children

- Consulted with experts in young child pain assessment (outside NF1) and identified two measures to further assess:
  - The Preschool Pain Scale (PSPS)
  - The Simplified Concrete Ordinal Scale (COS-S)
- Potential funding to validate these measures through existing NCI NTAP-PAIN PRO study

New members welcomed – email taryn.allen@nih.gov

Hearing and Communication Subgroup

- Heather Thompson, Vanessa Merker & Amanda Bergner
- Objective: To evaluate PROs related to speech, language & hearing
- Current focus: evaluate PROs related to hearing

Hearing and Communication Subgroup

The following are examples of PROs to evaluate:

- Speech, Spatial and Qualities of Hearing Scale
- Tinnitus Reaction Questionnaire
- Facial Clinimetric Evaluation Scale
- Voice Handicap Index

New members welcomed – email heather.thompson@csus.edu

Future Directions

- Continue review the general QOL scales and make recommendations.
- Finish review of disease-specific QOL scale and make recommendations
- Continue subgroups work for specialized domains.
- Update REINS website with recommended tools and methodologies
- Develop and use electronic measures

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If interested in being an active member of the PRO working group:
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