

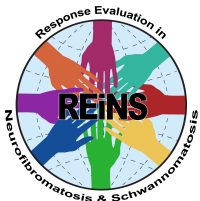
REiNS Patient Reported Outcomes Working Group

REiNS Committee Meeting 6/13/17

Ana-Maria Vranceanu, PhD
Massachusetts General Hospital, Harvard Medical School
&

Pam Wolters, PhD
National Cancer Institute

On behalf of the REiNS PRO group

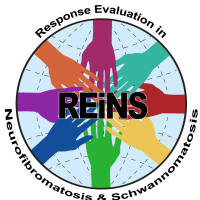


Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

REiNS PRO Working Group

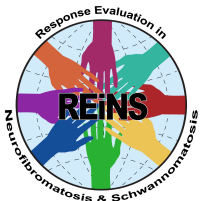
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Jim Tonsgard, MD
Ana-Maria Vranceanu, PhD
Karin Walsh, PsyD
Pam Wolters, PhD

National Cancer Institute
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NF Network
UNC-Greensboro
Guy's Hospital
Patient Advocate
MGH/Harvard
University of Pittsburgh
University Hospitals of Cleveland
Indiana University
National Cancer Institute
MGH/Harvard
Cincinnati Children's Hospital
California State University
University of Chicago
MGH/ Harvard
Children's National Medical Center
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

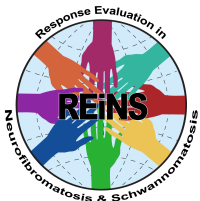
2) Physical Functioning

- Mobility
- Upper extremity

3) Disease Specific QOL

- NF1
- NF2
- Schwannomatosis

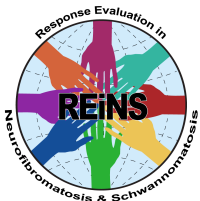
4) General QoL



Current Work of the PRO Group

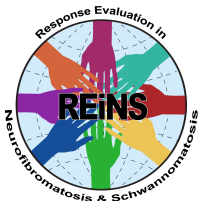
Core PRO Endpoint Domains (≥ 8 years)

- 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.



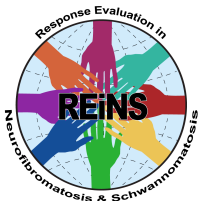
General QoL

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.



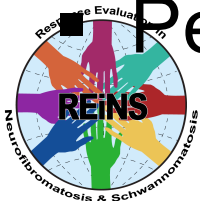
General QoL

- **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)



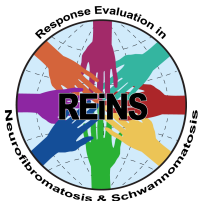
General QoL

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

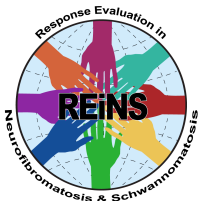
New members welcomed – email

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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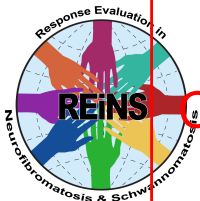
California State University

University of Chicago

MGH/Harvard

Children's National Medical Center

National Cancer Institute



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

Contact Pam Wolters at woltersp@mail.nih.gov or Ana-Maria Vranceanu at avranceanu@mgh.harvard.edu