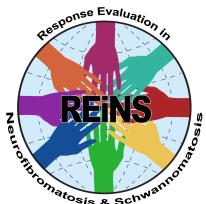


REiNS Patient Reported Outcomes (PRO) Working Group

REiNS Committee Meeting
December 4th, 2017

Heather Thompson, PhD CCC-SLP
California State University, Sacramento

On behalf of the REiNS PRO group



Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

REiNS PRO Working Group

Taryn Allen, PhD
Andrea Baldwin, CRNP
Amanda Bergner, MS CGC
Kim Bischoff
Tambra Dahlheimer
William Dudley, PhD
Rosalie Ferner, MD
Barbara Franklin, BS
Chris Funes, MS
Kathy Gardner, MD
Deborah Gold, MD
Susie Henley
Cynthia Hingtgen, MD, PhD
Staci Martin, PhD
Vanessa Merker, BS
Tena Rosser
Betty Schorry, MD
Heather Thompson, PhD CCC-SLP
Jim Tonsgard, MD
Ana-Maria Vranceanu, PhD
Karin Walsh, PsyD
Leader: Pam Wolters, PhD

National Cancer Institute
National Cancer Institute
Johns Hopkins University
NF Network
University of Minnesota
UNC-Greensboro
Guy's Hospital
Patient Representative
MGH/Harvard
University of Pittsburgh
University Hospitals of Cleveland
Guy's Hospital
Indiana University
National Cancer Institute
MGH/Harvard
Children's Hospital Los Angeles
Cincinnati Children's Hospital
CSU, Sacramento
University of Chicago
MGH/ Harvard
Children's National Medical Center
National Cancer Institute



REiNS PRO Working Group

- Patient Representatives
 - Current Member
 - Barbara Franklin
 - New Members
 - Krista Fredrick
 - Stephanie Reeve



Patient Reported Outcome

- “...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 *perception* of the severity of a specific symptom or disease.
 - Assess patient's *perception* of the impact of disease or treatment.



PRO Working Group

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



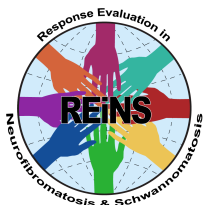
Core PRO Domains

- **Previous work** (2011 – 2016)
 - 1) **Pain**
 - Pain intensity
 - Pain interference
 - 2) **Physical Functioning**
 - Mobility
 - Upper extremity
- **2 PRO group publications summarizing this work**
(Wolters et al., 2013, 2016)



Core PRO Domains

- **Current work** (2016 – present)
 - 3) **Disease Specific QOL**
 - NF1 (e.g., *PedsQL NF1 module*)
 - NF2 (*NFTI-QOL and PANQOL*)
 - Schwannomatosis
 - 4) **General QOL**
 - Adults (e.g., *FACT-G*)
 - Children (e.g., *KINDL-R*)
 - Children through adults (e.g., *PedsQL generic core*)



PRO Subdomains

Current Work (2016 – present)

1. Pain assessment in young children
2. Communication



REiNS PRO

Communication Subgroup

Amanda Bergner

Simon Best

Ann Blanton

Patricia Ciavarelli

Kevin Franck

Barbara Franklin

Simon Freeman

Perez Gramajo

Chris Halpin

Simon Lloyd

Deborah Mawman

Vanessa Merker

Scott Plotkin

Ana-Maria Vranceau

Brad Welling

Pam Wolters

Leader: Heather Thompson

Columbia

Johns Hopkins

SUNY Cortland

University Buenos Aires

Harvard

Patient Representative

Manchester University

Argentina

Mass Eye and Ear Infirmary

Central Manchester University H.

Central Manchester University H.

MGH

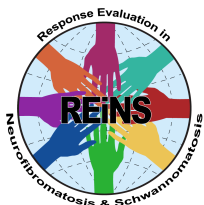
MGH

MGH

Mass Eye and Ear Infirmary

NCI

CSU, Sacramento



REiNS PRO

Communication Subgroup

- Background/Purpose
 - Individuals with NF may exhibit communication disorder (CD).
 - PROs give a voice to those with CD (American Speech-Language-Hearing Association [ASHA], 2015).
 - PROs help clinicians/researchers to document treatment outcomes related to communicative functioning.
 - ASHA indicates best practice must include patient perspectives (e.g., evidence maps; ASHA, 2017).



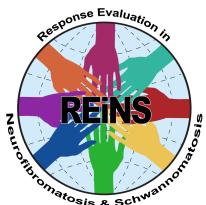
REiNS PRO

Communication Subgroup

- Objective: To evaluate PROs related to speech, language & hearing.
- Current focus: Evaluate PROs related to hearing.

Example PROs:

- Speech, Spatial and Qualities of Hearing Scale
- Client Oriented Scale of Improvement



REiNS PRO

Communication Subgroup

- Challenges:
 - CD is common in NF.
 - Impairments in speech, language and/or hearing may present barriers to obtaining patient perspective information.
 - Wording of items may be complex.



REiNS PRO

Communication Subgroup

- Benefits:
 - Useful for treatment trials for NF2.
 - Useful for trials that include patients with facial PNs.
 - Required for speech and language intervention: clinical practice and research.



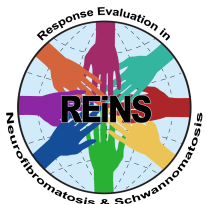
Future Work of the PRO Group

- PRO group
 - Complete the reviews and provide recommendations for general QOL scales.
 - Return to reviews of disease-specific QOL measures.
 - Prepare manuscript on QOL recommendations.
- Communication subgroup
 - Continue to review measures related to hearing.
- Young child pain subgroup
 - Work on attaining funding to explore the use of new young child pain measures in NF.



REiNS PRO Working Group

If you are interested in being an active member of
the PRO working group: Contact
Pam Wolters at woltersp@mail.nih.gov or
Ana-Maria Vranceanu avranceanu@mgh.harvard.edu



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