

Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

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Update from the Patient Reported Outcomes Working Group

REiNS 2021 Summer Meeting

Webinar

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on behalf of the REiNS PRO group



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Overview of Presentation

Purpose:

- To provide an update on PRO group work on measures used to evaluate the domain of disfigurement for patients with NF1
- To provide an update on PRO group work on measures used to evaluate the domain of tinnitus for patients with NF2

Definition of disfigurement

- Visible differences that can affect aesthetic, functional, or psychological well-being
- NF1
 - pNFs, cNFs, scoliosis, other bony issues, café au lait macules
- NF2
 - Orbital meningiomas, facial weakness, ptosis, muscle wasting
- Schwannomatosis
 - No symptoms identified

Definition of disfigurement

- Visible differences that can affect aesthetic, functional, or psychological well-being
- NF1
 - pNFs, cNFs, scoliosis, other bony issues, café au lait macules, short stature
- NF2
 - Orbital meningiomas, facial weakness, ptosis, muscle wasting
- Schwannomatosis
 - No symptoms identified

Current Goals

1. Validate a rating scale to assess tumor-related disfigurement (pNF and cNF)
2. Decide on measures of appearance concerns that would be applicable to tumor-related disfigurement



Item 2 – pNF Disfigurement

2. Is your _____ the most important disfigurement on your face or body?

Yes No

If yes – end of questions.

If no, please answer questions 3 and 4.

3. Where is your most important area of disfigurement on your face or body?

4. How disfigured is that area **today**?

0 1 2 3 4 5 6 7 8 9 10

Not at all
disfigured

Very
disfigured



Tumor-Related Appearance Concerns

- Derriford Appearance Scale (DAS)
 - How distressed do you get when you see yourself in the mirror/window?
 - I am self-conscious of my feature [tumor].
 - How feminine/masculine do you feel?
 - I avoid communal changing rooms.

Tumor-Related Appearance Concerns

- Center for Appearance Research – Valence Scale (CARVAL)
 - The way I look makes me feel good about myself.
 - I feel bad about my body and my appearance.
- Multidimensional Body-Self Relations Questionnaire (MBSRQ)
 - I like my looks just the way they are.
 - I don't care what people think about my appearance.

Challenges & Opportunities

- Identify measures that would be appropriate for:
 - Children/adolescents through adults
 - People with learning disabilities
 - People with NF tumors (vs other causes of disfigurement)

Challenges & Opportunities

- Identify measures that would be appropriate for:
 - Children/adolescents through adults
 - People with learning disabilities
 - People with NF tumors (vs other causes of disfigurement)
- Have validated tools that can document improvements in disfigurement as a secondary outcome in clinical trials



Future Directions

- Apply for funding to conduct a mixed-methods (qualitative and quantitative) validation study of the disfigurement tools
 - Get input from patients with pNF and cNF tumors through focus groups and cognitive interviews
 - Determine appropriateness of measures of tumor-related disfigurement and appearance concerns
 - Assess psychometrics of (modified) measures and appropriateness for NF clinical trials



Tinnitus PROMs

- There are challenges to assessing tinnitus with PROMs in NF2 trials
 - Many measures available; few are validated or used widely
 - PROMs are not used routinely as part of clinical care in Audiology
 - Item wording is not ideal for NF clinical trials

Example Items

“I wish someone understood what this problem is like”

“I have always been a light sleeper.”

Purpose

- To evaluate PROMs for the assessment of tinnitus in individuals with NF2

Measures Evaluated for the Domain of Tinnitus

Measures Evaluated	
THI	COSI - tinnitus
TRQ	STSS
TSQ	TAQ
TSI	TPFQ
TCQ	THSS
TFI	TCSQ
TQ	TBF-12

Measures Evaluated for the Domain of Tinnitus

Measures Evaluated	
THI*	COSI - tinnitus
TRQ*	STSS
TSQ	TAQ
TSI	TPFQ*
TCQ	THSS
TFI	TCSQ
TQ	TBF-12

- Most highly-rated measures as of April 2021.
- Need to complete a side-by-side review

Tinnitus Handicap Inventory (THI)

- To evaluate the impact of tinnitus on a person's daily life (*functional, emotional*)
- Adult normative data, can be used with children 10+
- Has been used in NF
- Good item content; psychometrics
- Short; feasible; available in 30+ languages

Example Items

“Do you feel that your tinnitus problem has placed stress on your relationships with members of your family and friends?”

“Do you feel that you can no longer cope with your tinnitus?”



Tinnitus Reaction Questionnaire (TRQ)

- To evaluate psychological distress caused by tinnitus (*emotional, physical*)
- Adult normative data; many published studies
- Has been used in NF
- Good psychometrics
- Short; feasible; available in multiple languages

Example Items

“My tinnitus has made me feel depressed”

“My tinnitus has led me to avoid social situations”

Tinnitus Primary Functions Questionnaire (TPFQ)

- To evaluate the effect of treatment on primary functions affected by tinnitus
- Adult normative data
- Has not been used in NF
- Good psychometrics
- Short; feasible; available in multiple languages

Example Items

"I am depressed because of my tinnitus"

"My emotional peace is one of the worst effects of my tinnitus"



Preliminary Recommendations of PROM for the Domain of Tinnitus

- Adults:
 - THI, TRQ or TPFQ depending on outcome of side-by-side reviews
- Children:
 - Tinnitus is not well-covered and is in need of further study
- Final PRO group recommendations for tinnitus are expected to be finalized in the Fall of 2021

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Q & A

- For researchers: What upcoming trials could we utilize to assess these disfigurement measures?
- For patient reps: would you find value in having a PROM for tinnitus to help inform your treatment plan?