



Response Evaluation In Neurofibromatosis Schwannomatosis INTERNATIONAL COLLABORATION

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Updates from the PRO Working Group: An Evaluation of Measures Used to Assess Anxiety and Depression for Individuals with Neurofibromatosis and Schwannomatosis

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Overview of Presentation

Purpose:

- To provide an update on PRO group work on measures used to evaluate the domains of anxiety and depression for patients with NF and SWN



Background

1. Previous work
2. Current direction



Background

- Individuals with NF1 have been shown to exhibit elevated levels of depressive symptoms (Talei-Khoei et al., 2017; Cohen et al., 2015; Wang et al., 2012) and anxiety (Wang et al., 2012).
- Higher rates of depression are associated with a higher number of medical visits per year (Wang et al., 2012).
- Depression, but not anxiety, has been shown to mediate the association between pain intensity and pain interference (Doorley et al., 2021).
- Results of a mind-body program showed improved symptoms of depression for patients with NF2 who are deaf (Carter et al., 2021).
- The FDA requires PRO measures to be used as part of the drug approval process.
- There is currently no single recommended measure for the assessment depression or anxiety for clinical trials in NF.



Depression

- ...characterized by depressive mood (e.g., sad, irritable, empty) or loss of pleasure accompanied by other cognitive, behavioral, or neurovegetative symptoms that significantly affect the individual's ability to function (WHO, 2023).

Anxiety

- Characterized by ... either general apprehension (i.e. ‘free-floating anxiety’) or excessive worry focused on multiple everyday events, [...concerning family, health, finances, and school or work], *together with additional symptoms such as muscular tension or motor restlessness, sympathetic autonomic over-activity, subjective experience of nervousness, difficulty maintaining concentration, irritability, or sleep disturbance* (WHO, 2023).

Current Goal of the REiNS PRO Group

- To decide on measures to assess symptoms of anxiety and depression for medical and psychosocial clinical trials in NF and SWN.



Challenges & Opportunities

- Identify measures that would be appropriate for:
 - Children/adolescents through adults.
 - People with learning disabilities.
 - Need to have a measure where the recall period is over a period of time, but not greater than 1 month.
 - Must assess general symptoms of anxiety and depression rather than specific types or diagnoses.



Challenges & Opportunities

- Have validated tools that can document improvements in symptoms related to anxiety and depression as a secondary outcome in clinical trials
 - The recall period needs to be long enough that anxiety and depression changes are still captured (one month).
 - Responses may be different if captured at home versus in a clinical setting.



Measures retrieved to date

Measure	Full Name	Domain Area*
PHQ-9	Patient Health Questionnaire	D
GAD-7	General Anxiety Disorder-7	A
CES-D	Center for Epidemiologic Studies Depression Scale	D
STAI, STAI-X2 STAI SF	The State-Trait Anxiety Inventory The State-Trait Anxiety Inventory (post-test) The State-Trait Anxiety Inventory (6-item short form)	A
BSI	Brief Symptom Inventory	B
HADS	The Hospital Anxiety and Depression Scale	B
QIDS	Quick Inventory of Depression Symptomatology	D
POMS	Profile of Mood States	B
SCL-20, SCL-90	The Symptom Checklist	D B
BASC-2	Behavior Assessment System for Children -2	A
SAFA	Self Administrated Psychiatric Scales for Children and Adolescents	A
DQ	Depression Questionnaire	D
HAM-A	Hamilton Anxiety Rating Scale (HAM-A)	A
BAI	Beck Anxiety Inventory	A

* (A) = anxiety, (D) = depression, (B) = both

Conclusions

- Future group work will focus on identifying PROs that support NF clinical trials.
- Continue to seek patient representatives who can provide guidance on our selections.

Discussion

- Do you feel that anxiety and depression are important next areas for the PRO group to focus on?
 - If yes or no, why or why not?
 - Other suggestions?
 - Adverse effects?
 - Sleep/fatigue?
 - Role participation?



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Q & A

