

Assessing Person-Centered Health Outcomes in Adults with Plexiform Neurofibromas (pNF)

These recommendations are offered as a starting point for consideration. They are not necessarily the best choices for every application and do not substitute for a comprehensive literature review.

Key Domains to Consider in pNF

Plexiform neurofibromas (pNFs) are a common complication of neurofibromatosis. Individuals with pNFs experience symptoms that negatively impact quality of life (QOL), including pain, organ dysfunction, compression and invasion of vital internal structures, leg length discrepancy, amblyopia, and the visible manifestation of the tumor affecting appearance. Patients with pNF experience significant symptom burden throughout their lives, making QOL an important clinical and research outcome for this population. Patient-reported assessments of **pain, stigma, anxiety, and social functioning (social activity/role participation)** can be used in both clinical care and research for monitoring and characterizing symptom burden and functional impact from NF1 pNF.

Suggested HealthMeasures for Primary Domains in pNF

Pain

Pain is a concern for patients with pNF across all ages. The PROMIS® Pain Interference measure assesses extent of pain experience and the effect of pain on daily activities. It is available as a 6-item short form or via computer adaptive testing (CAT).

Stigma

Disfigurement associated with pNF can impact appearance, body image, self-esteem, and social functioning. The Neuro-QoL Stigma measure assesses perceptions of self and publically enacted negativity, prejudice, and discrimination resulting from disease-related manifestations. It is available via an 8-item short form or via CAT.

Anxiety

Many patients with pNF express anxiety about disease progression and malignancy. The PROMIS Anxiety measure assesses fear (fearfulness, panic), anxious misery (worry, dread), hyperarousal (tension, nervousness, restlessness), and somatic symptoms associated with arousal (racing heart, dizziness). It is available as an 8-item short form or via CAT.

Social Functioning

Patients with pNF are concerned about their appearance/disfigurement, which may prevent them from normal social functioning. The PROMIS Ability to Participate in Social Roles and Activities measure assesses degree of involvement in one's usual social roles, activities and responsibilities, including work, family, friends and leisure. It is available via an 8-item short form or via CAT.

Summary

- Recommended primary domains for pNF research or care include pain, stigma, anxiety, and social activity/role participation
- Recommended secondary domains for pNF research or care include depression, physical function, fatigue, psychosocial illness impacts, and self-efficacy
- HealthMeasures offers brief, psychometrically sound measures for these domains in English and Spanish.
- Recommended assessment times include pre-treatment (baseline) and post-treatment.

Suggested Health Measures for Secondary Domains in pNF

The pNFs can impact many aspects of a person's physical, emotional, and social functioning, leaving other health status and quality of life domains significantly impacted. The following list the secondary domains addressed by patients with pNF:

Secondary HealthMeasures	Description
PROMIS Depression 8-item short form or CAT	Assesses negative mood, views of self, social cognition, decreased positive affect, and engagement.
PROMIS Physical Function 10-item short form or CAT	Assesses function of upper extremities, lower extremities, central regions, and activities of daily living.
PROMIS Fatigue 13-item short form or CAT	Assesses sensations the extent of fatigue experience and the effect of that fatigue on daily activities
PROMIS Psychosocial Illness Impacts – Positive 8-item short form or CAT	Assesses positive emotional and social outcomes of illness such as benefit-finding and meaning-making that can occur from confrontation with mortality.
PROMIS Psychosocial Illness Impacts – Negative 8-item short form or CAT	Assesses the direct negative emotional and social effect of illness, distinct from general emotional distress. (e.g., fear of condition worsening).
PROMIS Self-Efficacy: Manage Emotion 8-item short form or CAT	Assesses confidence to manage/control symptoms of anxiety, depression, helplessness, discouragement, frustration, disappointment, anger, stress and loss.
PROMIS Self-Efficacy: Manage Social Interaction 8-item short form or CAT	Assesses confidence in participating in social activities, getting help, and managing communication with others about their medical condition.
PROMIS Self-Efficacy: Manage Symptoms 8-item short form or CAT	Assesses confidence to manage symptoms and to keep symptoms from interfering with work, sleep, relationships or recreational activities.
PROMIS Self-Efficacy: Manage Daily Activity 8-item short form or CAT	Assesses confidence performing various activities of daily living without assistance.
PROMIS Self-Efficacy: Manage Medication /Treatment 8-item short form or CAT	Assesses confidence in managing medication and treatment schedules of 1) different complexity and 2) in challenging situations (travelling, when running out of medication, when encountering adverse effects).

Primary HealthMeasures for Adults with pNF

- PROMIS Pain Interference 6-item short form or CAT
- Neuro-QoL Stigma 8-item short form or CAT
- PROMIS Anxiety 8-item short form or CAT
- PROMIS Ability to participate in Social Roles and Activities 8-item short form or CAT

Learn More!

You can read about CATs and watch a videotutorial at the HealthMeasures.net website [here!](https://www.healthmeasures.net)

Assessment Times

Ideally, patient-report measures included in the set of primary domains (and secondary domains if patient burden is not too great) would be used at a variety of assessment time points, including pre- and post-treatment / intervention, both in clinical care and clinical trials.

Additional Information

The [www.HealthMeasures.net](https://www.healthmeasures.net) website includes more information about measurement selection, data collection tools, scoring, and interpretation. Its [Search and View Measures](#) tool includes access to all HealthMeasures described here. A Forum allows for questions and responses from the HealthMeasures community. The HealthMeasures team is also available for collaboration or consultation via help@healthmeasures.net.