Assessing Person-Centered Health Outcomes in Children and Adolescents 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, 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 quality of life 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 Interference
Pain was the primary concern in patients with pNF across all ages. The PROMIS Pain Interference measure assesses effect of pain on daily activities. It is available as an 8-item short-form or via computerized adaptive testing (CAT).

Stigma
Disfigurement associated with pNF can impact appearance, body image, self-esteem, and social functioning. The Neuro-QoL Stigma measure assess perceptions of self and publically enacted negativity, prejudice and discrimination resulting from disease-related manifestations. It is available as 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.

Peer Relationships
Patients with pNF are concerned about their appearance/disfigurement, which may prevent them from normal social functioning. PROMIS and Neuro-QoL Peer Relationships measures assess degree of involvement with one's peers in usual social roles, activities and responsibilities. Both measures can be administered by using an 8-item short form or via CAT. Both measures were validated on children with pNF and a crosswalk table is available to link both measures.

Summary

- Recommended primary domains for pNF research or care include pain, stigma, anxiety, and peer relationships.
- Recommended secondary domains for pNF research or care include depression, physical function, fatigue, psychosocial stress experiences, meaning and purpose, and positive affect.
- 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 Children and Adolescents with pNF

The pNFs can impact many aspects of a person’s physical, emotional, and social functioning, which means that other health status and quality of life domains can also be significantly impacted and represent secondary domains worth assessing. The following list the secondary domains addressed by patients with pNF:

<table>
<thead>
<tr>
<th>Secondary HealthMeasures</th>
<th>Description</th>
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<tr>
<td>PROMIS Depression 8-item short form or CAT</td>
<td>Assesses negative mood, views of self, and social cognition, decreased positive affect, and engagement.</td>
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<tr>
<td>PROMIS Mobility and Upper Extremity Function 8-item short forms or CATs</td>
<td>Assesses self-reported functioning of one’s upper extremities, lower extremities, central regions, and activities of daily living.</td>
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<tr>
<td>PROMIS Fatigue 10-item short form or CAT</td>
<td>Assesses sensations ranging from tiredness to an overwhelming, debilitating and sustained sense of exhaustion that decreases one’s capacity for physical, functional, social and mental activities.</td>
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<tr>
<td>PROMIS Psychological Stress Experiences 8-item short form or CAT</td>
<td>Assesses thoughts or feelings about self and the world in the context of environmental or internal challenges, including: feeling overwhelmed, perceived lack of control in managing one’s life, and cognitive-perceptual disruption.</td>
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<tr>
<td>PROMIS Meaning and Purpose 8-item short form or CAT</td>
<td>Assesses a child’s sense that life has purpose and there are good reasons for living.</td>
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<tr>
<td>PROMIS Positive Affect 8-item short form or CAT</td>
<td>Assesses a child’s momentary positive experiences, such as feelings and mood associated with pleasure, joy, elation, contentment, pride, affection, happiness, engagement, and excitement.</td>
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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 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.