Patient-reported outcomes

Progress toward speaking the patient's language

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Neurology® 2012;78:1813–1814

Increasingly, clinical trials are using patient-reported outcomes (PROs) as primary outcome measures, so as to improve the assessment of the overall effect of the intervention on the patient as a whole, rather than the more restricted assessment of the intervention on the condition itself. Consistent and reliable measurement of the effects of treatment for the patient is likewise becoming a larger part of clinical practice and reimbursement considerations. This is especially important for treatments that focus on reducing symptoms or improving function, rather than offering cure for a given condition. Measurement of the disease or treatment effect in a patient-centered way is especially critical in patients with neurologic conditions, since brain diseases, more so than other conditions, affect the entirety of who the patient is individually and in relationship to others.

Although recognized as important, measuring PROs can be difficult due to the complexity of the underlying condition, the more subjective nature of some important elements of outcomes, and the effort involved in obtaining these assessments. For some time, the primary approach to the problem of measuring PROs has been to develop disease-specific tools, either that measure a single construct or that measure multiple domains of health (often referred to as health-related quality of life measures).[1] These PROs are almost always developed using classic test theory, making them sample-dependent. There may be a trade-off between length of the instrument and its ability to discriminate among patients who may differ with respect to the effects of a disease or its severity. For example, it is challenging to develop a single instrument that can as meaningfully measure the patient-related effect of stroke on a patient with isolated visual deficits as it can for a patient with aphasia and hemiparesis. Further, disease-specific PROs can diminish the ability to understand and compare multiple conditions in a single patient or to compare interventions or treatments across different patient groups and different clinical trials.

One of the 3 focus areas in the NIH's 2002 roadmap for research in the 21st century was "Re-engineering the Clinical Research Enterprise." A key program in this area was the Patient-reported Outcomes Measurement Information System (PROMIS) program, which aimed to develop, calibrate, and validate common item banks for dynamic assessment of fundamental health domains relevant to many chronic conditions.[2,3] Substantial work has been done by the multicenter PROMIS group since the launch of this project (www.nihpromis.org).

The article by Cella et al.[4] in this issue of *Neurology*® reports a subsequent NINDS-funded project that draws on the PROMIS program to develop and test a PRO measure relevant to patients with several common neurologic conditions. The resulting tool, the Neuro-QOL (www.neuroqol.org), represents a major advance, not only because it is patient-centric and has good validity across these neurologic conditions, but because its Item Response Theory–based development makes it comprehensive, efficient, able to discriminate across severity levels, and amenable to computer-adaptive testing platforms. Item Response Theory is a method of scaling or assigning a numerical weight to an individual's response on a given item. It differs from typical summated rating scale construction methods in that it takes into account both item scaling (how difficult is the item) and subject scaling (the ability of the subject). Although it has been used most widely in the construction of computer-adaptive testing–based educational examinations (e.g., mathematical ability) it has been increasingly applied in health settings to measure more subjective constructs like health-related quality of life. Although more time-consuming to develop, a major advantage compared to traditional scaling methods is that the resulting measure is much less sample-dependent and can with fewer items better discriminate a precise ability level than can a classic summated rating scale.

Although further work remains to be done, the Neuro-QOL is an important leap forward in measurement of PROs in neurology. Neurology clinical

See page 1860

From the VA HSR&D Stroke Quality Improvement Research Initiative, Neurology, Indiana University School of Medicine; and Regenstrief Institute, Inc., Indianapolis, IN.

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trialists should be encouraged to evaluate the Neuro-QOL in a broader range of neurologic populations with varying disease severity, to compare the Neuro-QOL to more commonly used disease-specific outcome measures in the existing conditions tested (epilepsy, stroke, amyotrophic lateral sclerosis, multiple sclerosis, and Parkinson disease), to test the Neuro-QOL in patients with other neurologic conditions, and to determine important tool characteristics (e.g., minimally important difference) among patients with various neurologic conditions. The advent of computer-based testing platforms and office-based electronic medical records will also increase the opportunity for clinicians to quickly and reliably assess patient status and response to treatments in the outpatient setting. Embracing the ability to robustly yet efficiently measure PROs as we assess interventions in patients with neurologic disease will ensure that our clinical care and our research increasingly speaks the language of the patient.

DISCLOSURE

The author reports no disclosures relevant to the manuscript. **Go to Neurology.org for full disclosures.**

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