The Promise of Patient-Reported Outcomes Measurement Information System—Turning Theory into Reality

A Uniform Approach to Patient-Reported Outcomes Across Rheumatic Diseases

James P. Witter, MD, PhD

KEYWORDS

• PROMIS • Patient assessment • Validation • Universal

KEY POINTS

- The Patient-Reported Outcomes Measurement Information System (PROMIS) implements modern measurement theory and techniques to advance self- and proxy assessment of symptoms and health-related quality-of-life concepts.
- PROMIS enables improved measurement precision with less respondent burden by embracing item-response theory and computer-adaptive testing.
- PROMIS focuses on measuring universally relevant domains of health and disease to allow agnostic assessments across diseases and clinical settings facilitating meaningful cross-disease comparisons.
- By standardizing patient-reported outcome assessments, PROMIS supports the accumulation of data across settings, which enables meta-analysis and increases the amount of information that can be brought to the interpretation of the scores.

PROMIS® (a registered trademark of the US Department of Health and Human Services)—the Patient-Reported Outcomes Measurement Information System—is helping to facilitate an evolution in the science of patient/person self-assessment of experiences during health or disease. PROMIS represents a cooperative research

Disclosure Statement: The author has nothing to disclose. Opinions in this article are those of the author and not the National Institutes of Health/National Institute of Arthritis and Musculoskeletal and Skin Diseases.

Rheumatic Diseases Clinical Program, National Institute of Arthritis and Musculoskeletal and Skin Diseases, One Democracy Plaza, 6701 Democracy Boulevard, Suite 800, Bethesda, MD 20892-4872, USA
E-mail address: james.witter@nih.gov

Rheum Dis Clin N Am 42 (2016) 377–394
http://dx.doi.org/10.1016/j.rdc.2016.01.007 **rheumatic.theclinics.com**
0889-857X/16/$ – see front matter Published by Elsevier Inc.

program involving multiple academic medical centers, private research organizations, and numerous Institutes across the National Institutes of Health (NIH). It was designed to bring the most advanced measurement science to the development, evaluation, and standardization of item banks to measure patient-reported outcomes (PROs) of health-related quality of life (HRQL) across medical conditions. This system is the result of adopting and implementing concepts like item response theory (IRT) and standard setting (bookmarking), commonly used in educational testing, to measure and interpret health-related outcomes. PROMIS measures are applicable to be used as primary, secondary, or exploratory outcome measures in both adult and pediatric clinical research as well as to provide assessments of HRQL in patient care settings. This evolution in the assessment of PROs is occurring in many fields of medicine, not the least of which is rheumatology.[1]

The PROMIS initiative was one of the efforts of the NIH Roadmap (later Common Fund) initiative in 2004[2] designed to re-engineer the clinical research enterprise. The funding announcement laid out a new vision by noting that, "*The clinical outcomes research enterprise would be enhanced greatly by the availability of a psychometrically validated, dynamic system to measure PROs efficiently in study participants with a wide range of chronic diseases and demographic characteristics*." As noted, it established a collaborative working group between NIH and individual research teams throughout the United States to develop a measurement system and take it through various stages of growth and maturation (see later discussion, under New Science of Patient-Reported Outcomes).

Funding for this greater than 10-year initiative resulted from the recognition at the time that there was no common PRO language and no national standardized set of PRO instruments. Rather, what existed was a "Tower of Babel" approach for assessing PROs across diseases such as rheumatoid arthritis (RA), psoriatic arthritis, or systemic lupus erythematosus (SLE). Certainly, disease-specific outcome measures, especially those that include patient self-assessments, have been demonstrated to be useful for studies within the population in whom they were developed. However, such specificity has hampered the ability to easily and meaningfully compare the level of symptoms and other burdensome aspects of compromised health that make up "health-related quality of life" from one disease to another. Arguably, this lack of a common PRO language also hinders the ability to integrate and synthesize valuable PRO data into a better understanding of common pathogenic mechanisms that drive disease and adversely impact health. Moreover, a common language is required to assess PROs for patients with multiple chronic conditions.

NEW SCIENCE OF PATIENT-REPORTED OUTCOMES

Robust qualitative and quantitative studies, using a "mixed methods approach," are essential components for PRO development and validation according to modern measurement principles and current standards.[3–5] Despite this, few of the legacy or traditional PROs currently used in clinical medicine, including rheumatology, have been developed with this degree of rigor and attention, especially the inclusion of input from those living with and impacted by the conditions under study during the instruments' development. Many of these fundamental principles, especially those that relate to establishing the content validity of a developing PRO instrument, have been delineated in the US Food and Drug Administration's (FDA) PRO Guidance Document.[4] Those principles, illustrated in **Fig. 1**, require a series of iterative steps to ensure thorough psychometric and clinically focused validation.