

Editorial

More Evidence on the Validity of the Measurement Properties of PROMIS Computerized Adaptive Tests in Systemic Lupus Erythematosus



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Systemic lupus erythematosus (SLE) significantly affects different aspects of patients' health-related quality of life (HRQOL).¹ In 1998, Outcome Measures in Rheumatology (OMERACT) proposed the first Core Domain Set (CDS) for SLE, which included disease activity, organ damage, adverse events, HRQOL, and economic costs.² The SLE OMERACT working group (WG) was established in 2018 and includes 160 members representing over 25 countries in 5 continents. Currently the group is working on updating the CDS and will then proceed to evaluate the measurement properties of instru-

ments specific for each domain to form the new OMERACT SLE Core Outcome Set.³

Both physician evaluation and patient perceptions of their health condition and well-being are important and complement each other for a holistic health assessment. Studies have shown a considerable discordance between physicians' and patients' assessments of health status and priorities. Whereas physician assessments of disease activity, damage, and adverse effects of therapies are based primarily on clinical and laboratory findings, patients' assessment of their health can be ascertained with the use of different patient-reported outcomes (PROs). PROs can offer insight into a spectrum of domains such as fatigue, depressive symptoms, pain, and physical function among others.

Different generic and disease-specific HRQOL questionnaires have been developed and validated for patients with SLE.^{1,4} Commonly used generic questionnaires include the 36-item Short-Form Health Survey (SF-36),⁵ a legacy instrument, as well as the EuroQol 5-Dimensional questionnaire (EQ-5D), both commonly used questionnaires in SLE randomized controlled trials (RCTs)^{4,6} and observational studies.⁷ SLE-specific instruments have also been used, albeit less commonly, in SLE RCTs and research studies and include the Lupus Quality of Life (LupusQoL),⁸ SLE-specific Quality of Life Questionnaire (SLE-QoL),⁹ SLE Quality of Life Questionnaire (L-QoL),¹⁰ LupusPRO, and Lupus Impact Tracker.¹¹ PROs can also provide valuable data on physical health (eg, pain, physical function), mental health (eg, fatigue, cognitive function, depression, anxiety, sleep), and social interactions (ability to participate in social roles and activities, and social relations). Instruments that collect this data include the Beck Anxiety Inventory (BAI), Perceived Deficits Questionnaire (PDQ-20), Beck Depression Scale-II (BDI-II), the Assessment of Chronic Illness Therapy Fatigue Scale (FACIT-F),¹² and Epworth Sleepiness Scale (ESS),¹³ among others.

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The US Food and Drug Administration (FDA) as well as OMERACT require the use of instruments with evidence of their measurement properties (reliability, validity [face/content and construct validity] and discriminative validity) in patients with SLE. The FDA also acknowledges the importance of PROs in the approval process for new therapies in SLE. We have previously reported on the measurement properties of selected PROs for use in SLE RCTs.⁴ Specifically, in 2020 we studied the evidence of the measurement properties of 3 instruments on which data were available from longitudinal studies and particularly RCTs: SF-36, LupusQoL, and FACIT-F.⁴ All 3 instruments yielded evidence supporting their content validity, reliability (internal consistency, and test-retest reliability), construct validity (convergent and divergent), and longitudinal responsiveness, in addition to thresholds of minimal clinically important differences (MCID), and discriminative validity in patients with SLE.⁴ Similarly, we recently showed that EQ-5D full health state showed ability to distinguish drug from placebo and responders from nonresponders in SLE RCTs,¹⁴ and its attainment was linked to protection against damage accrual.¹⁵ These PROs can be used as secondary endpoints in SLE trials to support labeling claims of new interventions and/or treatment efficacy in SLE. The current work being conducted by the SLE OMERACT WG will extend the work to assess the measurement properties of other instruments that were not covered in this review.³ A recent study by Williams-Hall et al demonstrated evidence supporting the content validity of SF-36, Lupus QoL, FACIT-F, and novel patient-reported symptoms for use in patients with SLE and lupus nephritis.¹⁶

The Patient-Reported Outcomes Measurement Information System (PROMIS) was created by the National Institutes of Health, using item response theory, to facilitate the assessment of different HRQOL constructs including physical, mental, and social health in diverse populations, including the general population and individuals living with a variety of chronic conditions and diseases.¹⁷ PROMIS evaluation of a specific construct (eg, pain, cognition) usually requires fewer items for precision compared to other legacy instruments. PROMIS is also available in different formats, specifically, paper version short forms and computerized adaptive tests (CATs). In general, PROMIS CATs require 4 to 6 items for a precise measurement of a specific construct. Thus, PROMIS CATs may reduce the time required to capture a specific construct, taking into account previous answers of the participant and adjusting the number of required items accordingly.

The body of evidence on the measurement properties of PROMIS CATs in SLE is increasing over time. In this issue of *The Journal of Rheumatology*, Kasturi et al conducted a qualitative study to assess the relevance and clinical utility of PROMIS CAT in SLE.¹⁸ Adult patients with SLE followed at a tertiary care academic medical center participated in this qualitative study. The authors included a cohort of patients with a wide spectrum of demographic and clinical disease phenotypes. Participants completed the PROMIS CAT survey of 12 selected domains and were invited to rate the relevance of each domain using a 5-point Likert scale. The authors also conducted

single-gender focus groups and interviews where a total of 28 women and 4 men participated in 4 focus groups and 4 interviews. The interviews lasted an average of 35 minutes and were conducted in person or by telephone. In the survey, participants found the PROMIS domains to be highly relevant to their experience with SLE. The focus groups also confirmed the comprehensiveness of the selected PROMIS domains in reflecting the breadth and depth of the impact of SLE on their lives. Five of the 12 domains were ranked as the most important HRQOL domains: fatigue, pain interference, sleep disturbance, physical function, and applied cognition abilities. The male participants positioned anxiety in the top 5 most relevant domains.

Current PROs including PROMIS have been used primarily for research and in RCTs. The participants in the study by Kasturi et al¹⁸ advocated for the use of PROMIS CAT surveys in their medical care to allow a holistic health assessment. They found the PROMIS CAT survey to be “straightforward,” “easy,” and “self-explanatory.”¹⁸ Participants also indicated that the PROMIS CAT survey scores facilitated the assessment of their health longitudinally. In addition, they noted that the PROMIS CAT survey enhanced the positioning of the patient since it encouraged “self-reflection” and “promoted self-awareness and validation of illness,” as well as facilitated better communication with healthcare team members.¹⁸ Interestingly, participants in this study identified other domains to be relevant to patients with SLE that are missing from PROMIS, including body image, intimate relationships, and family planning and/or pregnancy concerns. Additional relevant suggestions from participants included (1) a “not applicable” option for some domains such as pain, (2) expanding the 7-day recall which was felt to be too short of an interval for SLE (although recommended by FDA), and (3) inclusion of free text boxes to allow patients to expand on their HRQOL needs.

Canadian data from a single SLE cohort of 277 patients who completed 14 domains of PROMIS CAT (physical function [v2.0], applied cognitive abilities [v2.0], applied cognitive general [v2.0], mobility [v2.0], pain behavior [v2.0], pain interference [v2.0], ability to participate in social roles [v2.0], satisfaction with social roles [v2.0], sleep disturbance [v1.0], sleep-related impairment [v1.0], fatigue [v1.0], anger [v1.0], anxiety [v1.0], and depression [v1.0]) against 7 legacy instruments (SF-36, LupusQoL, BAI, PDQ-20, BDI-II, FACIT-F, and ESS) provided evidence on the reliability and construct validity of PROMIS CAT in SLE.¹³ Test-retest reliability of PROMIS was evaluated 7 to 10 days from baseline and showed moderate to excellent intraclass correlation coefficient results. Construct validity was assessed using a multitrait-multimethod matrix approach comparing 10 domains of PROMIS with SF-36 and demonstrated moderate to strong correlations between domains of both instruments. The average time to complete all 14 PROMIS CAT domains was 11.7 minutes, whereas the average time required to complete the SF-36, LupusQoL, BAI, PDQ-20, BDI-II, FACIT-F, and ESS was 22.13 minutes (SF-36 alone took 5.3 mins and LupusQoL 4.4 mins).¹³ Each PROMIS domain took less time to complete than the corresponding domain on the legacy instruments with the exception of the

sleep-related impairment domain of PROMIS compared to ESS (87 and 72 seconds, respectively).¹³ Katz et al also demonstrated adequate responsiveness of the PROMIS short forms in patients with SLE. MCIDs were derived for all PROMIS short-form domains and all patient acceptable symptom states (PASS) were appropriate.¹⁹

In summary, the current study by Kasturi et al¹⁸ provides additional important evidence on the measurement properties of PROMIS CAT for use in patients with SLE as a generic tool, particularly content and face validity. At the same time, and similar to findings from other research,^{8,9,11,20} patients clearly highlighted the missing domains relevant to those living with SLE and not reflected in the PROMIS CAT or other generic tools, as described in previous studies.^{1,8,11} PROMIS provides a good alternative to other instruments, taking into consideration its different available formats of administration and the relatively short time required to complete the 12 domains. Other studies have reported feasibility of the PROMIS CAT in patients with SLE.²¹ Nevertheless, patients in this study highlight the difficulty in attributing symptoms to SLE or other associated comorbidities, stating that when answering survey questions, they reflect on their health in a holistic fashion.

This study also highlights the acceptance of patients with SLE for using PROMIS CAT as an appealing instrument. Nevertheless, further research is required to assess other PROMIS CAT measurement properties in patients with SLE, including its construct validity, responsiveness, and interpretability (eg, MCIDs). Future studies should also focus on the utility and integrity of PROMIS CAT in monitoring disease activity with direct involvement of patients by tracking PROMIS CAT scores longitudinally.

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