



Patient-Reported Outcomes Measurement Information System in Children with Crohn's Disease

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Objectives To assess the criterion validity and responsiveness of Patient-Reported Outcomes Measurement Information System (PROMIS) in a web-based cohort of children with Crohn's disease.

Study design We recruited children with Crohn's disease (ages 9-17 years) and their parents from the web-based Crohn's and Colitis Foundation of America Kids and Teens Study cohort. Upon entry into the cohort and 6 months later, children self-reported Crohn's disease activity, health-related quality of life, and PROMIS domains of pain interference, anxiety, depression, fatigue, and peer relationships.

Results Mean PROMIS scores for the 276 participating patients were worse among those with worse self-reported Crohn's disease activity (per Short Crohn's Disease Activity Index, $P < .005$ for all), Crohn's disease activity in the prior 6 months (per Manitoba Index, $P < .01$ for all), and health-related quality of life (per IMPACT-35, $P < .001$ for all). One hundred forty-three patients and their parents completed follow-up questionnaires, 75% of whom reported stable disease activity. Those with improved Crohn's disease activity reported improved PROMIS scores, and those with worsened Crohn's disease activity reported worse PROMIS scores for all domains except anxiety. All participants reported improved anxiety from baseline, but those with stable or worsened Crohn's disease activity reported less improvement ($P = .07$).

Conclusions PROMIS scores were significantly associated with Crohn's disease activity in a linear and clinically meaningful manner, and responded to change in Crohn's disease activity over a 6-month period. This supports the criterion validity and responsiveness of pediatric PROMIS. (*J Pediatr* 2016;174:153-9).

Patient-reported outcomes (PROs) are measures of how patients feel and function, obtained directly from patients without interpretation, capturing outcomes of importance to patients. PROs both complement standard primary outcomes, such as survival or physiologic measures, and provide primary outcomes that are necessarily patient-focused, such as patient function or quality of life (QoL).¹

The National Institutes of Health's Patient-Reported Outcomes Measurement Information System (PROMIS) is a set of nondisease specific instruments (one for adults and one for pediatric patients) assessing domains of physical, psychological, and social health, and QoL.² PROMIS instruments are unique in that they have been developed using modern measurement theory, including rigorous qualitative and quantitative methods.³ They are not disease-specific and are standardized to a reference population, allowing for comparison between different domains of health and across a wide range of chronic diseases.^{2,4} Pending additional clinical validation in children with chronic disease, PROMIS pediatric measures may serve as endpoints in clinical, observational, comparative effectiveness, and health services research.^{5,6} Researchers and clinicians will then be able to use these endpoints to identify previously unrecognized psychological, social, or functional health disorders, reveal correlations between these disorders and underlying chronic disease activity, and prompt appropriate interventions.⁷⁻¹⁰

When the onset of chronic disease is in childhood or adolescence, it can affect physical, psychological, and social development, as well as school performance, resulting in impaired or delayed achievement as an adult in interpersonal relationships, education, and employment.^{11,12} PROMIS pediatric instruments, which have been developed to assess these domains of health in children, have been studied in several disease states, including cancer, obesity, asthma, and nephrotic syndrome, and discriminate well among known groups of disease activity and severity.^{10,13,14} However, establishing the validity of PROMIS in children requires research in additional chronic diseases.

CCFA	Crohn's and Colitis Foundation of America
IBD	Inflammatory bowel disease
MID	Minimally important difference
NPTOT	Nonparametric tests of trend
PRO	Patient-reported outcome
PROMIS	Patient-Reported Outcomes Measurement Information System
QoL	Quality of life
SCDAI	Short Crohn's Disease Activity Index

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Children with inflammatory bowel disease (IBD) have been found to have lower QoL and social function than their healthy peers, and experience higher rates of depression than children with other chronic diseases.¹⁵⁻¹⁹ Crohn's disease has no definitive cure and commonly relapses, leading to intermittent disruptions to life and well-being, affecting a child's function, development, and QoL; thus, it is an important model of a relapsing pediatric chronic disease in which to further evaluate PROMIS.

In this study, we first aimed to evaluate the concurrent criterion validity of PROMIS (how PROMIS instrument scores relate to established measures of Crohn's disease activity and health-related QoL) by studying a national, prospective cohort of children with Crohn's disease. Second, we sought to explore the responsiveness of PROMIS instruments to change over time among cohort members who participated in a 6-month follow-up.

Methods

We performed cross-sectional analyses to evaluate associations between PROMIS measures, disease activity indices, disease-specific health-related QoL measures, therapy types, and remission status. We also performed exploratory longitudinal analyses to evaluate the associations between change in disease activity indices and the same QoL measures. The study protocol was reviewed and approved by the Institutional Review Board of the University of North Carolina at Chapel Hill.

The Crohn's and Colitis Foundation of America (CCFA) is a nonprofit, volunteer organization that funds, publishes, and advocates for IBD research, and provides support for patients with IBD of all ages. In 2011, the CCFA sponsored the development and maintenance of CCFA Partners, a web-based cohort of over 13 000 adults with self-identified IBD (ie, Crohn's disease and ulcerative or indeterminate colitis).^{20,21} Through a web-based portal, adults in the cohort complete research surveys every 6 months, receive research updates, and access CCFA educational resources.

In 2013, we launched a parallel, web-based cohort of pediatric patients with IBD and their parents, the CCFA Partners Kids and Teens Study. The purpose of this cohort is to create a long-term community of children with IBD engaged in research focused on the relationships between patient-reported exposures, health behaviors, and outcomes.²² We recruited patients with self-reported IBD and their parents via the CCFA by use of their website, e-mail rosters, various social media outlets, promotional efforts, and word-of-mouth at CCFA educational and fundraising events. Patients with at least one participating parent were eligible to enter the study. Patients and parents could access the cohort entry portal through the study website (www.ccfapartners.org) or through a promotional e-mail link. Enrollment began in August 2013 and remains ongoing. Parents completed the section of survey questions regarding

the child's diagnosis of Crohn's disease, demographic information, and family history. Children 9-17 years of age completed all symptom-related questions themselves, including PROMIS instruments specifically designed for child respondents. Six months after entering the cohort, participating patients and parents received e-mail reminders to complete follow-up questionnaires of the same disease-related questions and PROMIS instruments. For the present analyses, we included all patients aged 9 years or older with self-reported Crohn's disease who completed all initial surveys, including child-reported PROMIS instruments and parent-reported demographic information, by November 2013.

PROMIS

Participating patients completed 4 items (questions), from each of 5 selected PROMIS pediatric instruments: anxiety, depression, fatigue, pain interference (a measure of the consequences of pain on various aspects of life, including social, cognitive, emotional, physical, recreational activities, sleep, and enjoyment of life), and peer relationships (a measure of the quality of relationships with friends and other acquaintances).² We selected these instruments in collaboration with pediatric IBD specialists and experts in PROMIS methodology³ because they measure domains of health and health-related QoL affected by Crohn's disease in children. In an effort to minimize respondent burden and enhance long-term cohort retention, we chose to use 4-item short forms rather than longer forms or computer adaptive testing. Although 4-item short forms are less precise at the individual level, they can be effectively used in studies of moderate to large populations such as ours. PROMIS instruments are calibrated using a T-score metric with the mean of the original calibration population equal to 50, and the SD in the calibration population equal to 10.² Minimally important differences (MIDs) are the smallest differences in PRO scores able to detect a clinically meaningful change in the outcome that the PRO is designed to measure. Any difference in PRO scores smaller than the MID is likely due to measurement error instead of a true change in the outcome. Studies in adults suggest MIDs for many PROMIS items to be in the range of 2-6.²³ Though MIDs are not yet well established in PROMIS pediatric measures, new research using adolescent patients, parents, and physicians as judges of clinically important differences in scores, suggests MIDs of 2-3 for multiple pediatric PROMIS instruments.²⁴ Higher scores in any PROMIS domain indicate more of the domain being measured, therefore, higher scores for anxiety, depression, fatigue, and pain interference indicate poorer well-being, whereas higher scores for peer relationships indicate better relationships with peers and, therefore, better well-being.

Other Variables

We administered the IMPACT-35 questionnaire to assess health-related QoL, and we measured disease activity using

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