



Patient-Reported Outcomes in Adult Idiopathic Inflammatory Myopathies

Dana DiRenzo¹ · Clifton O. Bingham III¹ · Christopher A. Mecoli¹

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Abstract

Purpose of Review Idiopathic inflammatory myopathies (IIM) have considerable impact on patient symptoms and quality of life. We have reviewed the evolution of patient-centered care and use of patient-reported outcome measures (PROMs) for adults with IIM.

Recent Findings Use of PROMs in myositis care and research is limited, although the importance of incorporation into routine practice and trials has become increasingly recognized. Several key domains/measures have been identified including the patient global assessment of disease activity, physical function as measured by the health assessment questionnaire-disability index (HAQ-DI), Short Form Health Survey-36 (SF-36), or the Patient-Reported Outcome Measurement Information System® (PROMIS®) in adult IIM. Data are limited for these instruments concerning their reliability, content and construct validity, and responsiveness.

Summary Incorporation of the patient perspective into clinical care and research may be used to address the unmet/unaddressed needs of the patient living with myositis. Several ongoing projects aim to bring validated PROMs to the IIM community.

Keywords Idiopathic inflammatory myopathies · Patient-report outcomes · Patient-centered care · Health-related quality of life

Introduction

Adult idiopathic inflammatory myopathies (IIM) are a group of systemic inflammatory diseases which include dermatomyositis (DM), polymyositis (PM), and immune-mediated necrotizing myopathies (IMNM) among others. These conditions are typically characterized by muscle weakness but can also affect other organ systems including the skin, lungs, and joints. As a consequence, IIM are associated with significant impairment in health-related quality of life (HRQoL) [1]. While traditional outcome measures have focused on clinical assessments and laboratory testing initiated by the healthcare provider, patient-reported outcomes (PROs) originate from the patient. PROs are defined as any report of the status of a

patient's health condition that is conveyed directly by the patient, and the measurement of a PRO is thus a patient-reported outcome measure (PROM). Effectively, PROMs utilize validated questionnaires to turn a symptom into a quantifiable score, which can then be followed over time. Both in the USA and abroad, the importance of PROs and PROMs has been increasingly recognized over the last decade [2, 3].

The Food and Drug Administration (FDA) and European Medicines Agency (EMA) have strongly urged the use of PROMs to assess efficacy in clinical trials, and are working on how best to incorporate the role of the patient on scientific committees and advisory boards [4]. Furthermore, the importance of PROs has been recognized by the Centers for Medicare and Medicaid Services (CMS) as critical to the comprehensive evaluation of human health [5]. Several myositis organizations including the International Myositis Assessment and Clinical Studies Group (IMACS) and the Outcome Measures in Rheumatology (OMERACT) group have recommended the use of PROMs in clinical and observational studies to emphasize patient-prioritized outcomes. Until recent years, outcome measures in IIM were focused on clinical metrics (strength testing, electromyography, and serum creatine phosphokinase, CPK) but did not adequately capture patient-prioritized symptoms and

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✉ Christopher A. Mecoli
cmecoli1@jhmi.edu

¹ The Johns Hopkins University School of Medicine, 5200 Eastern Avenue, MFL Building, Center Tower, Suite 4100, Baltimore, MD 21224, USA

detriments to HRQoL. Within this context, a global assessment of disease activity was obtained from the physician, but not from the patient [6]. More recently, various PROs have been incorporated into the care of adult IIM patients. However, there remains a dearth of studies that systematically evaluate the status and trajectory of HRQoL using validated and relevant PROMs [7].

Part I

Brief History

Both OMERACT and IMACS have made considerable contributions to PRO development and implementation in IIM. OMERACT is an international network, established in 1992, with a mission to improve outcome measurements in rheumatology [8]. In 2011, the OMERACT Myositis Special Interest Group (SIG) was established. Its goal was to develop a set of core domains with respective measurement instruments that capture the experience of living with adult myositis from the patient perspective [9].

Initial qualitative work from the OMERACT SIG investigated which aspects of health were important to adult IIM patients. Over a 3-year period, eleven focus groups were conducted in three countries (Sweden, USA, South Korea) to elicit the experiences of adult patients living with IIM. Questions posed during the focus groups included “In what way has myositis changed your life?” and “Can you describe a typical day in your life?” Through qualitative analysis, several distinct themes emerged, including organ-specific symptoms (muscle, lung, skin, and joint), physical activity and function, emotional health (anxiety, depression), and social impacts (relationships, personal care) [10]. To reach consensus on which domains and subdomains were viewed as most important, three rounds of modified Delphi were conducted incorporating more than 500 patients from over 40 countries in multi-lingual surveys. These studies identified four broad domains—fatigue, pain, physical activity, and muscle symptoms, that were deemed as most important to be assessed in clinical trials of IIM (Fig. 1) [11]. In addition, lung, joint, and skin symptoms were felt to be mandatory in specific circumstances (i.e., clinical trials focusing on patients with amyopathic dermatomyositis, or anti-synthetase syndrome with interstitial lung disease would include PROMs of skin and lung, respectively).

Similarly, IMACs, an international multidisciplinary organization hosted by the National Institute of Environmental Health Sciences (NIEHS), comprised of healthcare providers and researchers, also proposed the incorporation of patient input into a set of core domains to be assessed in clinical trials [12]. The IMACS’ Core Set of Domains and Measures includes the patient and physician global activity scores, manual muscle testing (MMT), health assessment questionnaire-disability index (HAQ-DI), and laboratory muscle enzymes (CPK,

transaminases, lactate dehydrogenase, aldolase). In 2016, IMACs partnered with an international group of myositis experts and developed myositis response criteria for use in clinical trials which have been accepted by both the American College of Rheumatology (ACR) and the European League Against Rheumatism (EULAR) [13]. The patient global rating of disease activity is one of the six core set measures that comprise the response criteria. The response criteria, similar to the core set measures, are based on improvements (> 5–15%, > 15–25%, > 25–40%, > 40%) in physician global activity, patient global activity, MMT, HAQ, muscle enzymes, and extramuscular activity or disease activity score [13].

Part II

Selected PROMs in Myositis Patient Care and Research

We have reviewed several PROMs that have been most commonly utilized in IIM research: patient global assessment of disease activity and HAQ-DI, as endorsed by IMACS, the Medical Outcomes Study Short Form-36 (SF-36), the Nottingham Health Profile (NHP), the Arthritis Impact Measurement Scale-2 (AIMS2), the Patient Reported Outcomes Measurement Information System-29 (PROMIS-29), and the Myositis Activity Profile (MAP) (Table 1), among others [14].

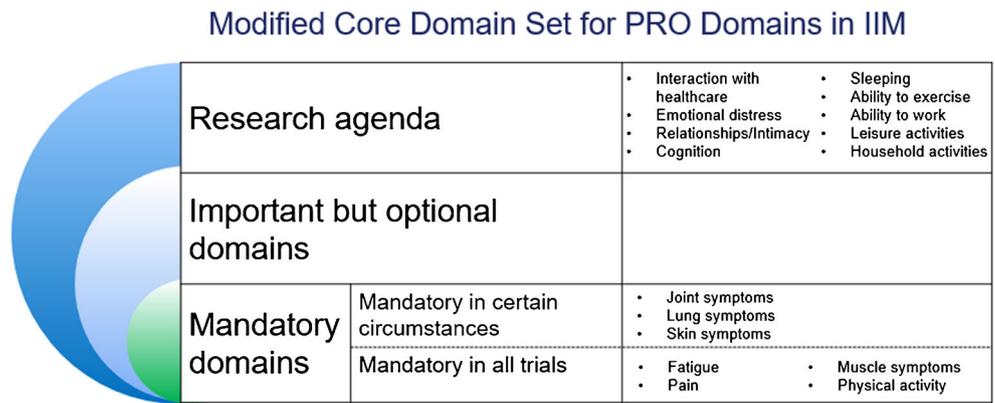
Patient Global Assessment of Disease Activity

The patient assessment of global disease activity is recognized by several myositis organizations and is intended to be the patient equivalent to the physician global rating of disease activity [15]. The IMACs question (form 03) is posed as “considering all the ways that myositis affects you, please rate the overall activity of your disease today by placing a mark on the line (10 cm visual analog scale).” The global disease activity score is recorded on a 10-cm visual analog scale or, alternatively, a 5-point Likert scale. While the common goal of this PROM is to garner a broad overview of myositis symptoms and impacts, the timeframe of interest may vary (present day to past 2–4 weeks). The scale takes less than 1 min to complete. There is no gold standard to assess criterion validity for the patient global assessment, and data on the ability to detect change is limited [15]. Consensus among myositis experts (rheumatologists, dermatologists, neurologists, statisticians) defines a clinically meaningful change as $\geq 20\%$ improvement [16].

Health Assessment Questionnaire-Disability Index (HAQ-DI)

The HAQ-DI is an instrument commonly used to evaluate physical function in rheumatic diseases. It is included by IMACs as one of the Core Set of Domains and Measures

Fig. 1 Modified OMERACT core domain set for patient-reported outcome (PRO) domains in adult IIM from Regardt et al. [11]



[15]. The HAQ-DI broadly evaluates functionality using a Likert scale across multiple categories including dressing/grooming, arising from a seated position, eating, walking, use of assistive devices, help from another person, hygiene, gripping, and activities [17]. Scores range from 0 to 3 and may be categorized by disability as mild (0–1), moderate (1–2), and severe (2–3). The HAQ was originally developed for use in rheumatoid arthritis, but it has been used frequently in IIM and has been helpful in identifying dysfunction not otherwise elicited through routine clinical measures. The HAQ has not been validated (content, construct) in adult patients with DM and PM; data is not available on the ability to detect change [15].

Short Form-36 (SF-36) [RAND-36]

The SF-36 is a widely used instrument that is also endorsed for the purpose of evaluation of physical function by IMACs for patients with IIM [18]. The SF-36 evaluates several subdomains including physical function, bodily pain, limitations due to physical health problems, limitations due to personal or emotional problems, emotional well-being, social functioning, energy/fatigue, and general health perceptions as well as a single item—perceived change in health [19, 20]. The eight domains may be used to derive a physical and mental health summary score, and a scoring manual is available for use online [21]. The construct validity of the SF-36 has been assessed in adult patients with IIM in which the physical functioning domain highly correlated with the HAQ-DI ($r = -0.71$) [15]. Content validity and the instrument responsiveness have not been reported in IIM [15].

Patient-Reported Outcome Measurement Information System® (PROMIS®)

PROMIS was developed by the National Institutes of Health (NIH) to provide highly reliable and precise instruments that may be used to measure common health symptoms and quality of life domains [22]. The instruments developed from large

item banks can provide a precise estimate of the severity of patient symptoms and disease impacts across a range of physical, emotional, and social health domains. The system was developed using item response theory and provides a score normalized to a standard population distribution with a mean of 50 and a standard deviation of 10 [23]. Fixed-length short forms and computerized adaptive testing administration is possible [24]. For example, the PROMIS-29 instrument is a 29-item fixed short form profile that covers 8 areas (physical function, anxiety, depression, fatigue, sleep disturbance, ability to participate in social roles, pain interference, and pain intensity). PROMIS measures have previously been validated in RA [25] and on a limited small scale in IIM [26–28].

Nottingham Health Profile (NHP)

The NHP is a HRQoL instrument that has been used less frequently in IIM. The NHP, a 38-item generic instrument, was implemented in a prospective observational study of patients with IIM, as compared to those with RA, osteoarthritis (OA), and osteoporosis [29]. The NHP is a two-part questionnaire; part I is most commonly used and special scoring algorithms, available for purchase, are required for derivation of domain scores [14]. Part I of the NHP addresses energy level, pain, emotional reactions, sleep, social isolation, and physical abilities, while part II focuses on the impact of disease on daily life, employment, home duties, social life, relationships, sex life, interests/hobbies, and vacations [30]. Analysis of content validity and instrument responsiveness have not been reported [2].

Myositis Activities Profile (MAP)

The MAP is the only IIM-specific PROM that has been developed to date [31]. The MAP was developed to better understand myositis disease activity and limitations of daily living [32]. The MAP was constructed for general use in adults with DM and PM and includes several sub-scales including Movement (8 items), Activities or Moving Around (4 items), Self-Care Activities (9 items), Domestic Activities (6 items),

Table 1 PROs previously implemented in myositis clinical trials

PRO instrument	Core domains evaluated/purpose	Subscales	Item number	Scoring
Global rating				
Patient Global Assessment of Disease Activity	Overall rating of myositis disease activity per the patient. *IMACS Core Measure	–	1	0–10 (10 cm visual analog scale; 10 indicates severe activity)
Physical function				
SF-36	Overall quality of life	Physical Function (10 items), Bodily Pain (2 items), Role Limitations Due to Physical Health Problems (4 items), Role Limitations Due to Personal or Emotional Problems (3 items), Emotional Well-Being (5 items), Social Functioning (2 items), Energy/Fatigue (4 items), General Health Perceptions (5 items); single-item Perceived Change in Health	36	Weighted sums of questions in each section are transformed into a 0–100 score; lower scores denote more disability. A Physical Summary component and Mental Health Summary component may be calculated using special algorithms (fee for service via a private company)
PROMIS-29	Overall quality of life	Physical Function (4 items), Anxiety (4 items), Depression (4 items), Fatigue (4 items), Sleep Disturbance (4 items), Ability to Participate in Social Roles (4 items), Pain Interference (4 items), And Pain Intensity (1 item)	29*	Raw score converted to standardized T-score with a mean of 50 and standard deviation of 10. Higher scores indicate more of the trait being measured
HAQ-DI	Physical function. *IMACS Core Measure	Dressing (2 items), Arising (2 items), Eating (3 items), Walking (2 items), Hygiene (3 items), Reach (2 items), Grip (3 items), And Activities (3 items)	20	The scores of the 8 sections are summed and divided by 8; mean scores range from 0 to 3 (0 = without difficulty)
AIMS2	Overall quality of life	Mobility, Walking and Bending, Hand and Finger Function, Arm Function, Self-Care, Household Tasks, Social Activities, Social Support, Pain, Work, Tension, and Mood	52	12 different subscales scored by summing the responses for all items in a scale; 0–10 (0 = normal function)
NHP	Overall quality of life	*2 parts; Part I most commonly used: Energy Level (3 items), Pain (8 items), Emotional Reactions (9 items), Sleep and Social Isolation (5 items), Physical Abilities (8 items)	38**	Special scoring algorithms required for purchase; only domain scores (not total) are reported. Each response within domain is a weighted for a total domain score of 100 (100 = worst health state)
Myositis specific				
Myositis Activities Profile (MAP)	Overall quality of life	Movement (8 items), Activities or Moving Around (4 items), Self-Care Activities (9 items), Domestic Activities (6 items), 4 single items—Social Activities, Avoid Overexertion, Work, Leisure	32	Each item is scored from 1 to 7 (7 = worst health state); median of all items defines the score for the subscale

SF-36 Stanford Short Form-36, PROMIS Patient-Reported Outcomes Measurement Information System, HAQ-DI Hospital Assessment Questionnaire-Disability Index, AIMS2 Arthritis Impact Measurement Scales-2. *Includes 4-item physical function short form, **Part I

Social Activities (single item), Avoid Overexertion (single item), Work (single item), and Leisure (single item) [32]. The MAP had a moderately high correlation to the HAQ for assessment of disease effect on well-being ($r=0.68$) when initially validated in adult patients with DM and PM in the USA [32]. However, qualitative studies conducted by the OMERACT

SIG determined that important symptoms of the patient experience were missing from the MAP including pain, fatigue, and impaired cognitive function [10]. An initial validation study noted moderate correlation with the HAQ ($r=0.69$) and moderate to excellent test-retest reliability (weighted kappa for each item ranged between 0.60 and 0.95) [32].

Arthritis Impact Measurement Scales-2 (AIMS2)

The AIMS2 was originally developed for use in arthritis to ascertain the overall status of quality of life and was used in the original validation of the MAP [31]. The AIMS2 is segregated into 12 subscales: mobility, walking and bending, hand and finger function, arm function, self-care, household tasks, social activities, social support, pain, work, tension, and mood [33]. Several of these subscales were used to determine construct validity for the MAP including the walking and bending, arm function, and work subscales (all $r > 0.50$) [31]. The AIMS2 represents a revised version of the AIM and additionally includes scales to evaluate arm function, work, and social support.

Use of PROMs in Recent Clinical Trials

Incorporation of PROMs in myositis research has been limited and inconsistent; however, the few randomized clinical trials in IIM have made efforts to include PROMs as outcomes. The patient global assessment visual analog scale and the HAQ-DI were used in the largest myositis randomized controlled trial (RCT) to date (Rituximab in the Treatment of Refractory Adult and Juvenile Dermatomyositis and Adult Polymyositis (RIM)) [34]. The RIM study used the definition of improvement (DOI) as endorsed by IMACS and defined as $\geq 20\%$ improvement in three of any six core set domains, which include the HAQ-DI and patient global, with no more than 2 worsening by $\geq 25\%$ [35]. Post hoc analysis of the RIM trial also used the HAQ-DI and patient global, as well as demographics, clinical/laboratory parameters, and myositis autoantibodies, to construct multivariable time-dependent proportional hazards models in prediction of treatment response [36]. The patient global was not associated with time to improvement (HR = 1.02, $p = 0.66$), and there was a trend of association for the HAQ (HR = 1.09, $p = 0.08$).

Clinical trials evaluating response of patients with DM and PM to adrenocorticotrophic hormone gel have also implemented the HAQ-DI and patient global [37]. At trial end, the patient global showed significant improvements, with an absolute change of 22.5% (10–30%) ($p = 0.003$), and the HAQ-DI showed a trend for improvements by 2% (0–13%) ($p = 0.098$). Other trials in adult IIM, including the study of lenabasum (NCT02466243) and tofacitinib (NCT03002649), have incorporated different PROMs such as the Beck Depression Inventory (BDI), 5-D itch scale, and PROMIS-29 Pain Interference and Physical Function (data forthcoming).

Part III

Patient Engagement and Operational Collection of PROMs in Patient Care

Outside of clinical trials, it remains important to incorporate PROMs in routine clinical practice and observational cohort studies. In order to successfully implement PROMs in these settings, they must be easy to administer and contain relevant content. As the health care system increasingly migrates to electronic systems, it is important to consider how PROs can be embedded within these systems for collection, scoring, and data displays to provide information to providers and patients in making shared medical decisions [38–40]. At the Johns Hopkins Division of Rheumatology, we have operationalized use of tablet computers for patients to complete PROMs in the waiting room. We then review values obtained from the patient during the appointment. We utilize the PROMIS collection of instruments, particularly the PROMIS Global Profile-29. Preference is given to PROMIS measures due to ease of use and translation of values into a T-score format (population mean, standard deviation of 50 ± 10 units). We display domain T-scores in a grid that are color-coded like a stop-light (green < 0.5 SD, yellow ≥ 0.5 SD but < 1 SD, red ≥ 1 SD). This allows rapid interpretation by the provider prior to entering the patient room and is easily understood by the patient (Fig. 2). We incorporate discussion about the patient’s scores and HRQoL into our encounter, which allows for a more concise and meaningful conversation. We can then focus on what HRQoL domain is less than ideal with

PROMIS Domain	T-Score	Color-coded status					Average or better
Physical Function	24.4						Worse 0 - <0.5 SD
Fatigue	65.3						Worse 0.5-<1 SD
Pain Interference	71						Worse 1 - <1.5 SD
Ability To Participate	37.7						Worse 1.5-<2 SD
Sleep Disturbance	54.3						Worse 2 SD or more
Anxiety	63.4						
Depression	62.2						

Fig. 2 Example of output from PROMIS-29 profile for an individual clinic patient. Each individual domain (e.g., Sleep) has an associated T-score reflecting the patient’s status compared to that of the general population

acknowledgement of those HRQoL domains that are going well. PROMIS T-scores are incorporated into the clinical note, and both our patients and providers have found implementation of PROMs into routine care very beneficial.

Future Directions

Despite the increasing utilization of PROMs in adult IIM research, additional research is needed to produce instruments with adequate content validity, construct validity, and discrimination. Work from the OMERACT SIG has identified the specific areas that are most important to patients—it is now vital to select instruments that encapsulate these items from the patients' perspective. Work is ongoing to identify such instruments and determine their measurement properties, both in longitudinal cohort studies and upcoming clinical trials.

Until myositis-specific instruments are developed, generic instruments to evaluate HRQoL may be implemented. Moving forward, standardizing use of PROMs for research and clinical practice will be important to avoid ambiguity when interpreting data between different cohorts and across trials. International organizations, such as IMACS and OMERACT, can play an important role in promoting the uptake of high-quality, validated PROMs in IIM research. This will aid in the aggregation of collected data, i.e., meta-analysis, which is necessary when studying a collection of rare diseases.

Conclusion

PROs, when used alongside clinical measures, add a unique perspective of disease activity as perceived by the patient. PROMs should be implemented in all patient care in order to improve the patient-physician relationship and shared decision making as the values of the patient and physician may not be equivalent. Refinement of myositis-specific instruments are necessary for high-quality care and research.

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Compliance with Ethical Standards

Conflict of Interest Dr. Bingham reports serving as an Executive Committee member for Outcome Measures in Rheumatology (OMERACT), which received hands-off funding from more than 20 pharmaceutical and research organizations. He receives no financial remuneration for this role. Dr. DiRenzo has nothing to disclose. Dr. Mecoli has nothing to disclose.

Human and Animal Rights and Informed Consent This article does not contain any studies with human or animal subjects performed by any of the authors.

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