

The Importance of Patient Input into Development of Outcomes in Idiopathic Inflammatory Myopathy



In this issue of *The Journal*, Mecoli, *et al* report the results of an international survey of healthcare providers and patients with idiopathic inflammatory myopathy (IIM) to examine and compare perceptions of disease features and effects¹. This work is part of an overall program to develop patient-reported outcomes in IIM, given that it is recognized that these have not been well studied to date². This study raises a number of important issues and has implications for future research.

This work follows the procedures described by the Outcome Measures in Rheumatoid Arthritis Clinical Trials (OMERACT) initiative. OMERACT is the acronym for an international, informally organized network initiated in 1992, which aimed at improving outcome measurement in rheumatology³. Using this methodology confers a number of important strengths to this work. It contributes to transparency by explicitly describing the process by which decisions are made throughout the process. It requires that decision making be data-driven, reducing the risk of the biases of the investigators compromising the results. The methods also mandate a broad input, by requiring the inclusion of patient research partners in the working group and involvement of researchers from at least 3 continents⁴. In this way, the likelihood of the results being valid and generalizable is increased. In the end, endorsement by OMERACT requires that the results pass the OMERACT filter: that is, do the resulting measures meet the criteria of truth, discrimination, and feasibility?³.

The field of rheumatology has shown considerable leadership in the area of patient-reported outcomes, a topic that has been reviewed⁵. The work of Mecoli, *et al* extends this to include patients with IIM¹. The same group have previously reported the results of qualitative research to explore the adequacy of current outcome measures in IIM and were able to demonstrate that outcomes of importance to patients with IIM were not assessed by current measures⁶. These additional outcomes included pain, fatigue, and impairment in cognitive function. The importance of these outcomes to patients with IIM has been confirmed by the

findings of the current work in this issue of *The Journal*¹. In addition, the work emphasizes the critical importance of engaging patients in the development of outcome measures. It should also be noted that despite the terminology *patient-reported*, the simple act of reporting an outcome does not mean that it is either important or relevant to patients. It remains necessary to ascertain which outcomes matter to the patients being studied.

Not surprisingly, priorities of patients may differ from those of their healthcare providers, because of the perspective granted by the lived experience of illness. The outcomes that are rated highly by care providers may be those that have been traditionally assessed, those that currently have validated assessment tools, or those perceived to be modifiable. There may be significant overlap, such as in this study, in which both patients and care providers rated muscle symptoms as the most important outcome¹. However, patients were more likely to highly rate fatigue, cognitive effects, and difficulty sleeping, while care providers were more likely to highly rate joint symptoms, lung symptoms, and dysphagia. These differences need to be used to identify important foci for future research.

Tory, *et al* have recently conducted an unrelated study that compared rankings of quality measures by patients with juvenile dermatomyositis and their families, and care providers⁷. Both groups agreed on the 5 most important quality measures: overall quality of life, timely diagnosis, access to rheumatology care, normalization of function/strength, and ability for self-care. However, there were also important differences. Patients and their parents rated overall quality of life as the most important quality measure, while it was ranked only fifth by the care providers. There were also a number of other measures that were ranked more highly by patients and their parents, including fatigue, pain, discontinuation of medication/steroids, and medication side effects. Again, the importance of asking patients about which outcomes matter to them is demonstrated.

This work by Mecoli, *et al* is an important step in the development and adoption of valid outcome measures for

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patients with IIM¹. It has the potential to affect both the conduct of clinical trials and the provision of clinical care. This study also identifies areas that will require additional research, because validated assessment of domains such as fatigue or cognitive impairment is currently not available for IIM. Finally, this work emphasizes the critical importance of directly involving patients, and where appropriate their caregivers, in outcomes research to ensure that all relevant domains are considered.

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