

Accelerated Cure Project for MS

March
2018



*Accelerating research towards a
cure for multiple sclerosis*

The Patient Perspective Makes a Big Difference

At some point, most people rely on their physician's recommendations and treatment for health maintenance or when they are sick. Doctors, in turn, rely on changes in [health status](#) (otherwise known as health outcomes) to determine a course of treatment, whether preventive or remedial. Health outcomes are classified according to the source from which they are collected. A physician reported outcome is one collected by a physician in the course of clinical care (for example, the physical exam, imaging scans or lab tests). A patient reported outcome (PRO) is one directly reported by the patient who experienced it. PROs are most often factors that matter most to the patient, such as symptoms, functioning and quality of life. Although physician reported outcomes are useful in assessing disease activity and severity, they do not help clinicians fully understand the true impact of illness on patients. For example, multiple sclerosis affects a wide variety of things, including employment status, social and family relationships, sexual satisfaction, pain, fatigue, enjoyment of life, vision, bladder and bowel control, cognition, as well as emotional well-being. These profound effects on patients' quality of life (which can influence the patient's adherence to treatment) are often not captured by the physician as a part of routine clinical care. PRO and physician reported outcomes measure different components of a patient's well-being and should ideally be used together to assess a patient's health status and to provide individualized treatment.



PRO data can help physicians identify minimal changes in disease activity which may otherwise go unnoticed, thus improving their assessment of symptoms and enabling them to provide better quality of care and encourage adherence to treatment plans. This is information that might otherwise be lost in

brief check-ups or in a patient’s inability to communicate aspects of their condition as they progress through the course of illness. In addition, consideration of PRO data in the course of clinical care can



foster stronger physician-patient relationships, encourage patients to increase ownership in managing their disease, and help physicians address patient concerns directly. In research, PROs provide useful information whenever the patient perspective enters the equation. For example, PROs are used in clinical trials to measure the risk and benefit of new drugs and therapies, and also play a significant role in determining when a study ends. Once a medication is FDA-approved PROs become an important tool to guide patients and clinicians to make the best treatment decisions possible.

PROs measure one or more specific aspect(s) of the patient experience from their perspective. These aspects are called “constructs”. The most commonly used PRO questionnaires focus on what is commonly referred to as [health-related quality of life](#), which are the aspects of quality of life that are related to health. This includes symptoms, functioning, emotional well-being, satisfaction with care/treatment, as well as knowledge of or adherence to therapy. PRO data can be collected in a variety of ways, for example through questionnaires, patient logs, or patient interviews. These methods of collection are referred to as “instruments”, “measures”, “scales” or “tools”. Regardless of the method, data should be provided directly by the patient whenever possible. Questionnaires that measure a single construct are referred to as “unidimensional”, and those that address multiple constructs are “multi-dimensional”. Questionnaires can be paper-based or electronic (administered via computer, tablet or smart phone). All have standardized scoring which provides consistent measure of the patient’s health status. Of note, PRO data collected from an interview can only be considered “patient reported” if the interviewer recording the patient responses is not confounding the data in any way by adding their own observations or opinions.



Typically, PRO instruments must undergo extensive validation and testing before they are used. They can be generic (designed to apply to a wide variety of patient groups) or disease-specific (developed specifically to assess the impact of a particular disease). The [SF-36](#) is an example of a generic questionnaire that many MS patients may recognize. The [Multiple Sclerosis Quality of Life Inventory](#) (MSQLI) consists of 10 individual scales, providing both generic and MS-specific quality of life measures.

With the exception of conditions that rely on patients’ reports (such as depression), PRO data is not widely used in the clinical setting. Despite the potential for broad benefit, clinicians’ main concerns are that additional data collection would be burdensome. PRO questionnaires may add costs that some

providers may find difficult to absorb. Interestingly, some feel that PROs are not necessary given they have already established a good relationship with their patients. In this case, clinicians believe their assessment of the treatment benefits is consistent with the patient's experience, which may not always be the case. PRO data also have some inherent limitations. It can be difficult to collect. Sometimes patients don't fully complete instruments, leading to missing data (if it cannot be obtained on follow-up). Surveys rely on patient recall, which may result in inaccurate information. In addition, patient responses may vary depending on cultural, ethnic, language, or educational differences among respondents. However, even with these limitations in mind, PROs provide essential input in both the clinical and research settings.

The [Patient-Centered Outcomes Research Institute](#) (PCORI) is an independent, nonprofit organization in Washington, D.C. that provides funding for comparative [clinical effectiveness research](#) and works to influence clinical and healthcare research funded by others to be more patient-centered. PROs are the core of their mission. PCORI was founded on the premise that patients, their families, and clinicians should have reliable information to decide which treatment or other care option is best for them. One of their primary goals is to increase the amount and quality of PRO data available to support health decisions.



As discussed on our [June 2017 newsletter](#), [iConquerMS™](#) is a Patient Powered Research Network, funded in part by PCORI, developed to advance and accelerate patient-centered research in MS. Its participants complete surveys on a number of topics through a secure portal, including demographics, MS symptoms and MS-specific PRO instruments (the [PROMIS® Global Health Survey](#), and the [Neuro-QoL Adult Short Forms](#)). iConquerMS enables all people living with MS to participate in research by providing their data, while effectively removing the main barrier that prevents the use of PRO data in MS research by making it readily available to investigators (either by providing access to the existing database of information, or by collecting new data from iConquerMS members). This collaboration between people affected by MS and the research community is part of an effort to transform MS research, improve the quality of care for MS patients and may one day play a significant role in finding a cure. If you haven't already done so, please consider [joining the iConquerMS community](#) today and add your voice to the thousands already driving research forward!

