

A Closing Thought

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Two Decades of Change for PROs

How patient-reported data collection has been transformed since 1987.

Patient-Reported Outcome (PRO) measures and data collected directly from the patient have certainly changed in the past 20 years. The world of PROs looks very different today than it did when we first developed our electronic diary methods for collecting data from patients in clinical trials in 1987. PROs have grown more important to drug development, more scientifically robust, and more technologically sophisticated. Here are some of the trends I've seen over the past two decades:

PROs become central. PRO measures have become ever more central to clinical development. Several factors have driven this: increased importance of indications in which end points are fundamentally subjective (e.g., pain); increased focus on chronic illnesses that may be managed or ameliorated by treatment rather than cured, making how patients feel more important; and attention to patient experience by formulary managers, payors, and regulators (particularly outside the United States).

PROs become focused. The focus of PRO measures is shifting from broad quality of life assessments to measures of signs and symptoms, which may be disease specific.

Treatment aims most directly to cure or mitigate disease or relieve its symptoms. Thus, signs and symptoms are core outcome targets. Treatment-related changes in quality of life are less direct—downstream consequences of these impacts on disease process. As PROs have become more focused on direct consequences of disease and treatment, they have more often served as primary end points in clinical trials.

Emergence of psychometrics. Perhaps the most important development has been greater attention to psychometrics, the science of assessing patient experience. Where development of PRO measures was once ad hoc and unsystematic, it is now increasingly formal and science based. We think harder now about what we need to measure, how it relates to the way treatment is expected to work, and what claims the PRO



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measures will support. Qualitative work is increasingly done to ensure that the intended measure taps the full range of patient experiences and questionnaires are subjected to rigorous quantitative statistical tests.

PROs go electronic. These days when we read about PROs, it's most often prefixed by an 'e.' Electronically collected PRO data (ePRO) has become an important methodology in clinical trials. As the technology and science have matured, the advantages of ePRO assessment have become compelling. Data gathered directly from the patient benefits from the operational advantages of electronic capture, such as its ability to present the right assessment at the right time, to handle skip patterns within assessments, and to impose data standards and run data edits right at the time of entry. So, with evidence that screen-based ePRO measures are equivalent to their paper predecessors, ePRO is rapidly replacing pPRO.

PROs move closer to patients' experience. With a substantial body of research showing that memory is a flawed instrument for capturing accurate data, clinical researchers are striving to reduce reliance on retrospective recall and moving to capture data closer to the real-time point of experience, often by using diaries. This is facilitated by the increasing sophistication of ePRO instruments, which allow patients to complete assessments easily, while automatically documenting the time of completion. This allows PRO assessments to capture more of the patients' real-life experience, and to do so more faithfully.

PROs have, indeed, come a long way. Not that we don't still have a ways to go: Psychometric science is still not always integrated in clinical planning; PRO end points in trials are not always clearly linked to end-point models linking disease, treatment, and outcome; and ePRO instruments are often used as electronic versions of paper measures, without fully leveraging what the technology is capable of. But we've come a long way. □



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