

Patient-Centeredness in Clinical Outcomes Assessment

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KEY POINTS . . .

Patient-reported outcome (PRO) measures are not always patient-centered since they may not have been developed with patient input; they may not be outcomes that patients find important.

Patient-reported outcomes are not the only patient-centered outcomes. In fact, sometimes they are not the most patient-centered outcomes.

Preferred outcomes may differ between subpopulations of persons with the same disease and can change depending on an individual's life situation, age, or other factors.



The following article is part of a regular feature highlighting local student chapter activities and research talents. In this piece, we draw attention to patient-centeredness in clinical outcomes assessment.

Background

There are many different types of health outcome measures (see Table) that can be classified by the type of information collected (e.g., survival) or how the information is collected (e.g., patient or clinician report). Regulatory and health technology assessment (HTA) bodies, along with professional organizations, have begun labeling certain outcomes as “patient-centered,” “patient-relevant”, or “patient-oriented.” There are inconsistencies in how these terms are used, what they mean, and how they are different from traditional outcomes, so there is confusion about what a patient-centered outcome is. This brief introduction highlights several key points to consider when evaluating the patient-centeredness of outcome measures used in past research or determining endpoints for future studies.

For an outcome measure to be “patient-centered,” it must measure something that patients identify as being most important to them. In this context, depending on the etiology of the disease, “patient” can also include caregivers. A variety of methods can be used to identify which outcomes are important to patients. These may include focus groups, patient interviews, or open-ended surveys. Collaborating with patient advocacy organizations or online patient communities helps researchers identify heterogeneous patient samples.

Patient-Reported Outcome Measures Are Not Always Patient-Centered

There is often the assumption that patient-reported outcome (PRO) measures are “patient-centered” by virtue of being reported by the patient. However, we often ask patients questions to gather information on outcomes and other endpoints that are unimportant to the patients themselves. Others might assume that because a PRO has been “validated” or published

in the peer-reviewed literature that it is “patient-centered.” However, validation is not relevant to patient-centeredness. For example:

1) *PRO measures may not have been developed with patient input; they may not be outcomes that patients find important.* To determine the questions to ask patients, measure developers often consult the peer-reviewed literature or seek clinician input. While a critical step to determine what has been studied in the past and helpful to determine important aspects of the condition, these sources may neglect the symptoms that patients find most important. For example, researchers and clinicians interested in creating a “core set” of outcomes for rheumatoid arthritis neglected fatigue as an outcome important to patients. It was not until patients were invited to participate, ten years after the first development meetings, when fatigue was identified as an important outcome of rheumatoid arthritis and has since become “central to our understanding of the effects of inflammatory arthritis.” [1,2] Early versions of these measures were considered “validated” and are available in the published literature. However, these PRO measures were *not* patient-centered.

2) *Language may not reflect the vernacular used by patients (see Marquis et al. [3] for example of how patient feedback can modify word choice).*

“For an outcome measure to be “patient-centered,” it must measure something that *patients* identify as being most important to them.”

Patient-reported outcomes are not the only patient-centered outcomes. In fact, sometimes they are not the most patient-centered outcomes. Over the past several years, the Food and Drug Administration (FDA) has convened patients and patient

Table. Types of Measures

Type	Description	Example
Clinical measure	Laboratory measure or a physical sign that does not require interpretation (e.g., a biomarker) [8].	CD4 count
Clinician-reported outcome (ClinRO)	Based on a report that comes from a trained health care professional after observation of a patient's health condition. A ClinRO measure involves a clinical judgment or interpretation of the observable signs, behaviors, or other physical manifestations thought to be related to a disease or condition. ClinRO measures cannot directly assess symptoms that are known only to the patient (e.g., pain intensity) [8].	Pitting Edema Measurement Scale
Morbidity	Condition of being diseased [9]	Disability indexes
Mortality	Death [9]	Overall survival; cardiovascular death
Observer-reported outcome (ObsRO)	Based on an observation by someone other than the patient or a health professional. This may be a parent, spouse, or other nonclinical caregiver who is in a position to observe and report regularly on a specific aspect of the patient's health. An ObsRO measure does not include medical judgment or interpretation [8].	Parent version of the Asthma-Related Anxiety Scale
Patient-reported outcome (PRO)	Based on a report that comes from the patient about the status of a patient's health condition without amendment or interpretation of the patient's report by a clinician or anyone else. Symptoms or other unobservable concepts known only to the patient can only be measured by PRO measures [8].	Pain severity or nausea
Performance outcome (PerFO)	Based on a task(s) performed by a patient according to instructions that is administered by a health care professional. Performance outcomes require patient cooperation and motivation [8].	Timed 25-foot walk test

advocates as part of their Patient-Focused Drug Development (PFDD) initiative [4]. The purpose of these meetings is to understand patient perspectives on symptoms that matter most to them, along with perspectives on impact on daily living and treatment options. During the Huntington's disease meeting, patients reported unsteady gait/trouble walking as one of their top three most significant symptoms [5]. In this case, a performance outcome measure with a clinician observing walking ability is more patient-centered than a patient-reported outcome measure on something else.

Importantly, preferred outcomes may differ between subpopulations of persons with the same disease and can change depending on an individual's life situation, age, or other factors [6]. For example, during the FDA's PFDD meeting on lung cancer, two outcomes emerged as very important: survival and quality of life. Patients who prioritized survival tended to be younger, have children, and have fewer symptoms. Alternatively, patients who were older and/or desired to end life peacefully

prioritized quality of life as equally or more important than prolonged survival [7].

Moving forward

Given current emphasis on patient-focused drug development, biopharmaceutical companies should anticipate increased emphasis on "patient-centeredness" in regulatory and reimbursement decision making in the future. To meet these needs, patient-centered outcome measures should be incorporated into trials being planned today. New and legacy instruments alike should be evaluated to determine whether they measure those things patients say are important to them. Decision makers should encourage these changes, but also recognize that given the amount of time clinical development plans take to execute, it may be some time before patient-centered new drug applications become a reality. In parallel, patient and research organizations should ensure that their registries and other data-collection efforts incorporate patient engagement activities in order to include questions on outcomes that patients prioritize. Finally, graduate programs in outcomes research

should incorporate curricula on patient-centeredness and outcomes assessment.

References

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Additional information:

For additional information on clinical product development, see the Report of the ISPOR Clinical Outcome Assessment Emerging Good Practices Task Force summary on page 23.

For further information on ISPOR Patient Centered Special Interest group, go to <https://www.ispor.org/sigs/PatientCentered.aspx>