Global Spine Journal
2021, Vol. 11(2) 266-268

DOI: 10.1177/2192568220978979
journals.sagepub.com/home/gsj

A Guide to the Language of Clinical Outcome Assessment

Joseph R. Dettori, PhD[1], Daniel C. Norvell, PhD[1], and Jens R. Chapman, MD[2]

> Not everything that can be counted counts. And not everything that counts can be counted.
>
> —Albert Einstein

Introduction

The results of clinical studies of spine surgery are reported utilizing various clinical outcomes measures. These include clinician-based outcomes (CBOs) and patient-reported outcomes (PROs). CBOs often include elements of surrogate and performance-based outcomes (PerfO). PROs can be general or disease specific. On occasion, components of either CBOs or PROs are combined to form composite outcomes (Figure 1). It is important to recognize the differences among these outcomes, and their strengths and weaknesses, in order to best interpret study results and conclusions. We will briefly consider each in this issue of Science in Spine.

Clinician-Based Outcomes Measures: Are You Happy With Your Patient's Progress?

Historically, clinicians have attempted to determine a patient's outcome by measuring attributes believed to be associated with well-being. Clinician-based outcomes refer to an array of tests and measures that assess the result of healthcare interventions from the *perspective of the clinician*. Most CBOs involve a clinical judgment or interpretation of observable signs, behaviors, or other manifestations related to a disease or condition. They stand in contradistinction to patient-reported outcomes measures (PRO); those outcome measures that reflect the patients' perception of their symptoms, functional ability, and quality of life.

CBOs often include laboratory, radiographic or physiologic measures that act as *surrogate outcomes*. A surrogate outcome does not measure the clinical benefit of primary interest in and of itself, but rather is expected to predict the primary clinical benefit (or harm).[1] Surrogate outcomes are attractive in that they are often cheaper, quicker and easier to assess. However, they are not always valid as a substitute for the outcome of interest. An example of a surrogate outcome for osteoporotic fracture is bone mineral density. To determine the validity of a surrogate outcome, consider the following[2]:

- The treatment effect on a surrogate ought to be associated with a treatment effect on a final outcome. The stronger the association, the better.
- The association between surrogate and final outcome ought to be consistent.
- There ought to be biologic plausibility of the relation between surrogate and final outcome.

CBOs may also include *performance-based outcomes (PerfO).* PerfOs are outcomes in which the patient is assessed but no evaluator judgment affects the measurement. The patient's performance is based on a defined task that is quantified in a specific way that does not rely on judgment to determine the measurement. An example is the Timed Up and Go (TUG) test or the 10 Meter Walk Test (10MWT) following surgery for cervical myelopathy.[3] Clinicians administering and monitoring the performance of PerfO tasks do not apply judgment to quantifying the performance.

Patient-Reported Outcomes: Is Your Patient Happy With His/Her Progress?

Patient-reported outcomes are questionnaires or instruments that patients complete by themselves or, when necessary,

[1] Spectrum Research, Inc., Steilacoom, WA, USA
[2] Swedish Neuroscience Institute, Seattle, WA, USA

Corresponding Author:
Joseph R. Dettori, Spectrum Research, Inc., PO Box 88998, Steilacoom, WA, USA.
Email: joe@specri.com

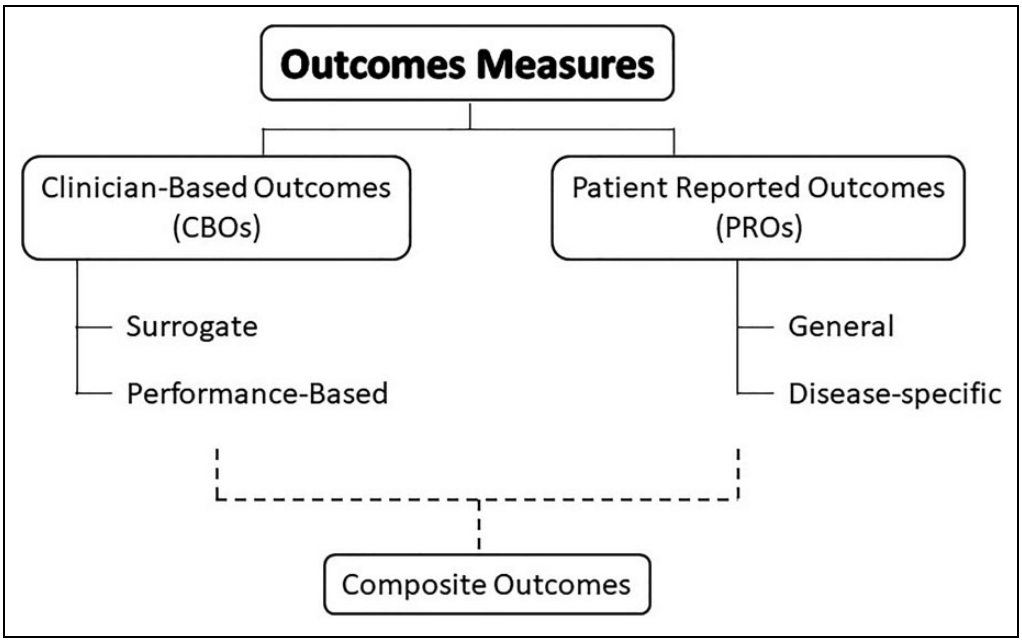

Figure 1. Clinical outcome measures.

others on their behalf to obtain information in relation to functional ability, symptoms, health status, health-related quality of life, and results of specific treatment strategies.[4] The patient's health status comes directly from the patient and is not interpreted by a clinician.

It is increasingly recognized that traditional clinician-based outcomes need to be complemented by measures that focus on the patient's concerns in order to evaluate interventions and identify whether one treatment is better than another. Interest in PROs has been fueled by an increased importance of chronic conditions, where the objectives of treatment are to restore or improve function while preventing future functional decline.[4,5] The Food and Drug Administration (FDA) encourages the appropriate use of PROs in regulatory studies as part of their Strategic Priorities and their commitment to "Partner with Patients."[6]

PROs are classified as either general or disease-specific measures. *General measures* are designed to assess health-related quality of life across different disease states and across different demographic and cultural subgroups. An example is the SF-36.[7] An important limitation of general measures is that they tend to be less responsive than specific measures of health-related quality of life to changes in health status[8] and therefore less likely to detect the effects of a specific intervention.

Disease-specific measures focus on aspects of health that are specific to a disease (e.g., cervical myelopathy), an injury (e.g., compression fracture), an anatomical area (e.g., the lumbar spine), or a population of interest (e.g., the elderly). The advantage of disease-specific measures is their ability to detect small but important changes that occur over time in the particular disease studied.[9,10]

Composite Outcomes: A Legitimate Measure?

A composite outcome combines several outcomes into a single endpoint, one in which the occurrence of any of the outcomes is considered an endpoint event. It is often used in studies testing new treatment strategies. An example would be a composite outcome of any complication whether the complication was serious or not. The main motivation for composite outcomes is to increase statistical efficiency. Using composite outcomes increases the observed endpoint event rate, reduces the number of participants needed in a clinical trial, takes less time and reduces costs. However, one must be cautious in interpreting results using composite outcomes. Often, investigators will combine components with varying clinical importance, which can make a treatment seem more effective or less harmful than it really is. For example, the least clinically important component included in the composite outcome may drive the size of the treatment effect, thereby overestimating its effectiveness. It is important for investigators to report data for all composite components in order to assist the reader's understanding of which components are most responsible for the treatment effect.

Key Points for Each Outcomes Type

Type of outcomes	Definition	Examples
Clinician-based outcomes (CBOs)	Tests and measures that assess the health status of patients from the *perspective of the clinician.*	• Spinal Cord Independence Measure (SCIM) • Functional Independence Measure (FIM)
Surrogate outcomes	Alternative outcomes that substitute for the outcomes of interest to the patient and are expected to predict the primary clinical benefit or harm.	• Bone mineral density (BMD) • Successful fusion
Performance-based outcomes (PerfOs)	A performance measure that assesses patients without a rater judgment impacting the measurement.	• Timed Up and Go (TUG) • 10 Meter Walk Test (10MWT)
Patient-reported outcomes (PROs)	Health status is reported directly by the patient and is not interpreted by a clinician.	• Pain Visual Analog Scale (VAS) • Oswestry Disability Index (ODI)
General PROs	Measures designed to assess health-related quality of life across different disease states, demographics, and cultural subgroups.	• 36-Item Short Form Health Survey (SF-36) • European Quality of Life (EUROQoL)
Disease-specific PROs	Measures that focus on aspects of health that are specific to a disease, injury, anatomical area or population of interest.	• Neck Disability Index (NDI) • Oxford Spinal Stenosis Score (OSSS)
Composite outcomes	One that combines several outcomes into a single endpoint. The occurrence of any of the outcomes is then considered an endpoint event.	• Any of the following complications: Dural tear, delusion, hematoma, meralgia, pulmonary embolism, misplacement of pedicle screw[11]

References

1. Tufanaru C. Surrogate outcomes. *JBI Database System Rev Implement Rep*. 2016;14(11):1-2.
2. Ciani O, Buyse M, Drummond M, Rasi G, Saad ED, Taylor RS. Time to review the role of surrogate end points in health policy: state of the art and the way forward. *Value Health* 2017;20(3):487-495.
3. Cheng YS, Chien A, Lai DM, et al. Perturbation-based balance training in postoperative individuals with degenerative cervical myelopathy. *Front Bioeng Biotechnol*. 2020;8:108.
4. Norvell DC. Patient-reported outcomes measures. In: Chapman JR, ed. *Spine Outcomes Measures and Instruments*. AOSpine International; 2007:39-41.
5. Byrne M. Cancer chemotherapy and quality of life. *BMJ*. 1992; 304(6841):1523-1524.
6. US Food and Drug Administration. Value and use of patient-reported outcomes (PROs) in assessing effects of medical devices. CDRH Strategic Priorities 2016-2017. Accessed November 5, 2020. https://www.fda.gov/about-fda/cdrh-patient-engagement/clinical-outcome-assessments-coas-medical-device-decision-making
7. Ware JE Jr, Sherbourne CD. The MOS 36-item short-form health survey (SF-36). I. Conceptual framework and item selection. *Med Care*. 1992;30(6):473-483.
8. Guyatt GH, Feeny DH, Patrick DL. Measuring health-related quality of life. *Ann Intern Med*. 1993;118(8):622-629.
9. Patrick DL, Deyo RA. Generic and disease-specific measures in assessing health status and quality of life. *Med Care*. 1989;27(3 Suppl): S217-S232.
10. Wright JG, Young NL. A comparison of different indices of responsiveness. *J Clin Epidemiol*. 1997;50(3):239-246.
11. Inose H, Kato T, Yuasa M, et al. Comparison of decompression, decompression plus fusion, and decompression plus stabilization for degenerative spondylolisthesis: a prospective, randomized study. *Clin Spine Surg*. 2018;31(7):E347-E352.