Introduction:
A patient reported outcome (PRO) is any report that comes directly from a patient about how they function or feel in relation to a health condition. PROs are often used in clinical trials to evaluate the effectiveness of medical products (e.g., drugs, medical devices, biological products, etc.). This call will provide you with an introduction to PROs and PRO instrument development.

Objectives: At the end of this activity and research club teleconference, participants will be able to:

- Define patient-reported outcomes and distinguish them from other clinical outcome assessment attributes (e.g., clinician reported outcomes, observer-reported outcomes, etc.)
- Identify a patient-reported outcome (specific to their rare disease) that would be suitable for use as a clinical trial endpoint.

Pre-Read:

Additional Readings and Resources (Optional)


FDA Clinical Outcome Assessment Qualification Program.


Pre-Conference Call Activity:
An important step in the process of defining and developing PROs is identifying a concept of interest. The concept of interest is typically related to how a patient feels or functions (e.g., fatigue, ability to walk without stopping, etc.). Consider your respective rare diseases and think about what concepts of interest you want your PRO to focus on. Be prepared to share and discuss your thoughts and questions with the group.
Glossary of Terms (from the FDA)

Defining a clinical outcome assessment (COA): Clinical outcome assessments (COAs) measure a patient’s symptoms, overall mental state, or the effects of a disease or condition on how the patient functions. COAs can be used to determine whether or not a drug has been demonstrated to provide treatment benefit. Treatment benefit can also be defined in terms of a safety benefit compared to other treatments. A conclusion of treatment benefit is described in labeling in terms of the concept of interest, the thing measured by the COA.

- Clinician-reported outcome (ClinRO) — A ClinRO is 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).

- Observer-reported outcome (ObsRO) — An ObsRO is a measurement based on an observation by someone other than the patient or a health professional. This may be a parent, spouse, or other non-clinical caregiver who is in a position to regularly observe and report on a specific aspect of the patient’s health. An ObsRO measure does not include medical judgment or interpretation. Generally, ObsROs are reported by a parent, caregiver, or someone who observes the patient in daily life. For patients who cannot respond for themselves (e.g., infants or cognitively impaired), we encourage observer reports that include only those events or behaviors that can be observed. As an example, observers cannot validly report an infant’s pain intensity (a symptom) but can report infant behavior thought to be caused by pain (e.g., crying). For example, in the assessment of a child’s functioning in the classroom, the teacher is the most appropriate observer. Examples of ObsROs include a parent report of a child’s vomiting episodes or a report of wincing thought to be the result of pain in patients who are unable to report for themselves.

- Patient-reported outcome (PRO) — A PRO is a measurement based on a report that comes from the patient (i.e., study subject) about the status of a patient’s health condition without amendment or interpretation of the patient’s report by a clinician or anyone else. A PRO can be measured by self-report or by interview, provided that the interviewer records only the patient’s response. Symptoms or other unobservable concepts known only to the patient (e.g., pain severity or nausea) can only be measured by PRO measures. PROs can also assess the patient perspective on functioning or activities that may also be observable by others.

- Performance outcome (PerfO) — A PerfO is a measurement 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. These include measures of gait speed (e.g., timed 25 foot walk test), memory recall, or other cognitive testing (e.g., digit symbol substitution test).