BACKGROUND

- Outcome assessments are used to define efficacy endpoints when developing a therapy for a disease or condition.
- Primary outcomes in clinical trials may be assessed by endpoints relating to survival, biomarkers, and clinical outcome assessments (COAs).
- CDAs consist of clinician-reported outcomes (ClinROs), observer-reported outcomes (ObsROs), patient-reported outcomes (PROs), and performance outcomes (PerfROs), as defined by the Food and Drug Administration (FDA).
- The extent of use of each type of outcome as primary endpoints in clinical trials has not been well described in the literature.

ClinROs

- 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).

ObsROs

- An ObsRO is a measurement based on an observation by someone other than the patient or a health care professional. This may be a parent, spouse, or other nonclinical 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 patients), 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.

PROs

- 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 responses. Symptoms or other unobservable concepts known only to the patient (e.g., pain severity or nausea) can only be measured by PRO measures. PRO measures can also assess the patient's perspective on functioning or activities that may also be observable by others.

PerfROs

- 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).

OBJECTIVE

- To assess the extent of use of endpoints on survival, biomarkers, ClinROs, ObsROs, PROs, and PerfROs as primary endpoints in confirmatory studies of new drugs approved from 2011 through 2015.