Paper 1: Clinical Outcome Assessments (COAs): A Conceptual Foundation
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Abstract
Developing therapies for diseases where no therapy has yet been developed or for disease manifestations not previously evaluated by existing therapies will likely call for developing new and improved efficacy endpoints. Efficacy endpoints are based on specified assessments of patients and must be well defined and have adequate measurement properties to demonstrate the benefits of a treatment. How to develop new assessments for use in endpoints, or evaluate the utility of existing assessments is not always clear.

An initial step is clearly identifying and describing the meaningful aspect of how patients feel or function in their typical lives, or on survival, that is the intended benefit. This aspect is called the meaningful health aspect (MHA). When it is not practical or optimal to directly evaluate the selected health aspect, a bodily function or feeling that is thought a sub-component and more practical to measure may be identified and called the concept of interest (measured COI). The measured COI may be identical to the MHA when it is feasible or necessary to do so. Procedures are then developed to measure the COI. Also necessary is to fully define the circumstances and manner of use of the assessment, called the context of use.

Assessments have identifiable attributes that affect the measurement properties of endpoints. These attributes include whether patient motivation influences the measurement, whether, and whose, judgment can also influence the measurement, and whether the assessment is directly or indirectly related to the meaningful aspects of feelings and functioning. Recognition of the specific attributes of an assessment aid in directing efforts at defining, standardizing, and refining the assessment to improve the measurement properties. This paper discusses these important concepts that apply to all types of assessments used in efficacy endpoints.

Paper 2: Developing and Evaluating Clinician-Reported Outcome (ClinRO) Instruments to Assess Treatment Benefit: Good Measurement Principles
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Paper 2: Developing and Evaluating Clinician-Reported Outcome (ClinRO) Instruments to Assess Treatment Benefit: Good Measurement Principles
Abstract:
This paper describes good measurement principles and practices for developing and evaluating Clinician Reported Outcome (ClinRO) instruments. A ClinRO instrument assesses a patient’s health condition as judged...
and reported by a trained health care professional. Three main types are in use: 1) ratings; 2) readings; and 3) clinician global assessments. ClinRO instruments are appropriate when the most informative measurement of a patient’s health status is best made by a clinician. This can include situations where patients are unable to self-report on their own health status, or an assessment can only be performed by a professionally trained person.

Good measurement practices in developing and evaluating ClinRO instruments include: 1) defining the context of use; 2) identifying the concept of interest; 3) identifying and evaluating the relationship between the concept(s) of interest and the meaningful health aspect intended as the treatment benefit; 4) documenting content validity; 5) evaluating measurement properties; 6) considerations regarding interpretation of results based on the measurements; and 7) considerations in implementation of ClinROs in clinical trials.