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Editorial

Patient Voice in Clinical Trial Outcome Assessments

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Since the early 90s, there has been an increasing interest in incorporating clinical outcome assessment (COA) in clinical trials to measure how a patient feels or functions when describing the treatment benefit (both positive or negative) of an intervention. In 2009, the FDA issued the Patient Reported Outcome (PRO) Guidance which lays out the criteria for establishing the 'fit for purpose' of a COA, if that COA is to be used along the regulatory pathway (i.e., support approval or labeling). The principles elucidated in the PRO Guidance establish the gold standard for incorporating a COA, and provides the basis for the reliability, validity and interpretability of the COA data collected from a clinical trial on a registration path.

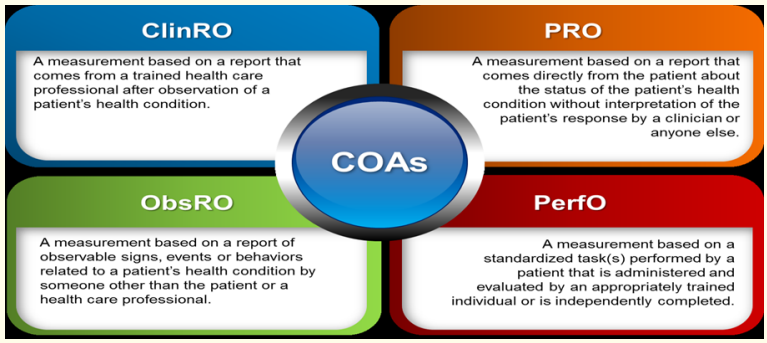
However, not all COAs are used along the regulatory pathway. It could be that researchers or academics are interested in only publishing the results of a study in which COAs have been included. In this case less rigor could be used to establish the 'fit for purpose' of the endpoint used in the trial.

Despite the presence of a body of work which provides a framework that can be used to establish the 'fit for purpose', the suggested guidelines are often ignored by companies engaged in developing a medical product; or researchers/academics interested in conducting epidemiological studies. Regardless of which pathway (i.e., regulatory or publication) is chosen, ignoring existing guidelines, can result in delays in approval or in the publication of important data, as submissions are rejected without the proper 'fit for purpose' evaluation of the COA.

The purpose of the short report is to provide some basics that can be used to incorporate a COA as an endpoint, primary or secondary, in a clinical trial, which can help to prevent delays in obtaining an optimal response to the planned submission.

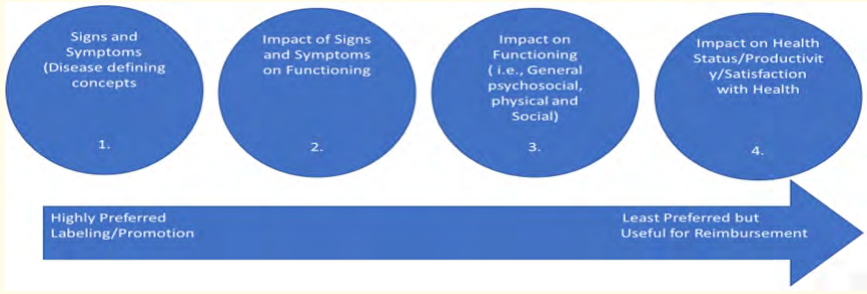
COA scan be assessed using four distinct types of COAs, see Figure 1. COAs can be disease specific or generic. Disease specific are often meant to capture effect of treatment/intervention on the symptoms (how the patient feels) and the impact of symptoms on patient function. Disease specific scales are usually unidimensional. Generic are multi-dimensional and capture the impact of the intervention on the health status or utility (for subsequent cost-utility analyses) of the patient. This hierarchy is shown in Figure 2.

The roadmap to COA selection is given in Figure 3. The three steps to the selection or development of an appropriate COA is to: 1-Understand the disease or condition to be studied; 2-Conceptualize the treatment benefit appropriate for that disease or condition, and 3-the selecting, modifying or developing the COA. For example, understanding the disease or condition (step 1) includes the comprehension of (a) disease natural history, (b) characteristics of patient subpopulations, (c) current clinical practice and therapeutic landscape and (d) patients' and caregivers' perspectives and values. Likewise, when choosing an appropriate COA which measures the concept of interest within the disease or condition, it is important that we define the COA (see figure 1), conduct a search to see if the COA exists and needs to be modified or if a new COA must be developed. Examples of each category is provided in Figure 3.



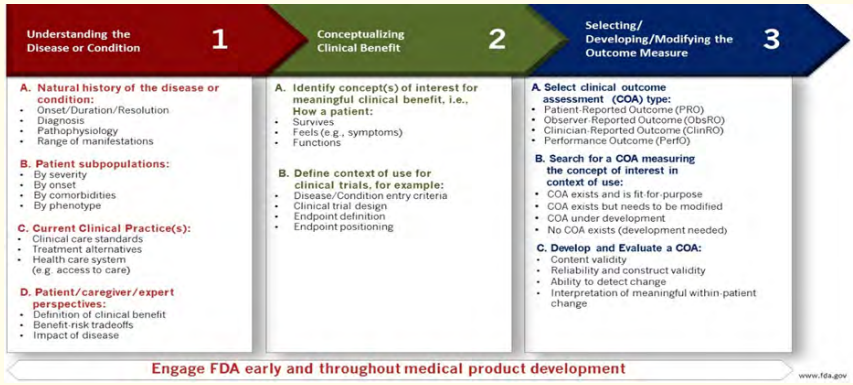
Source: PFDD Public Workshop Guidance 3 Discussion Document.

Figure 1: Types of COAs.



Source: RDhanda 'Why COAs' internal presentation.

Figure 2: Hierarchy of COA.



Source: PFDD Workshop Guidance 3 Discussion Document.

Figure 3: Roadmap to COA Selection/Development for Clinical Trials.

It is also important to understand the purpose that the COA will serve. Is the COA intended to be used within the regulatory pathway, or is it meant for publication? The answer to this question will define the approach that can be used for a 'fit for purpose' evaluation of the selected COA. The regulatory pathway requires patient/caregiver input based on a formal study. The publication pathway is less rigorous and can be based on psychometric methods to develop a COA based on patient response to a broader set of items or questions. Observing these requirements will help to return a positive outcome to any planned submission.

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