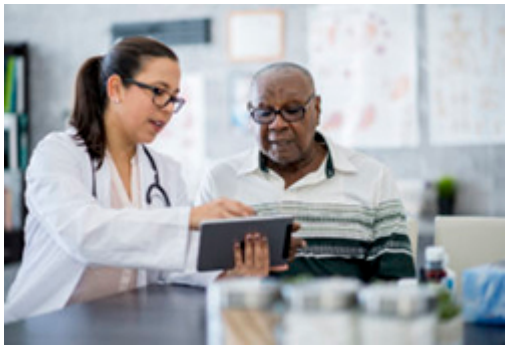
# What are Clinical Outcome Assessments (COAs) and Can They be Used to Support Approval and/or Labeling Claims?



The patient voice is recognized as one of the most critical sources of data in drug development, and patients play an increasingly important role in these efforts by teaching us about their experience with their condition and its impact. A common way sponsors can leverage the patient experience is by utilizing a clinical outcome assessment (COA). A COA is an assessment that describes or reflects how a patient feels, functions, or survives. Such an assessment can be a patient-reported outcome (PRO) measure, observer-reported outcome (ObsRO) measure, clinician-reported outcome (ClinRO) measure, or a performance outcome (PerfO) measure. **Alexander Varond** chaired a session on this topic in June 2020 at the Drug Information Association's Annual Meeting. Slides from his presentation can be found **here**.

FDA plans to issue a guidance that will provide patient-focused approaches and methods to consider in the selection and/or development of COAs. This future guidance, known as Patient-Focused Drug Development (PFDD) Guidance 3, is one piece of FDA's plan to develop a series of four PFDD-specific guidances for stakeholders on how to collect and utilize patient experience data in drug development. We initially discussed this plan and background on patient experience data **here**. In the meantime, FDA has described a "roadmap to COA selection/development for clinical trials" **here**. This roadmap sets forth how to obtain an understanding of the disease or condition, conceptualize clinical benefit (i.e., how a patient feels, functions and survives), and how to select, develop and modify a COA. In Guidance 4, FDA will discuss how to incorporate COAs into endpoints for regulatory decision-making. FDA issued a discussion document related to the forthcoming Guidance 4 **here**.

As background, a COA may support approval of a product if it is a "well-defined and reliable" assessment (21 CFR § 314.126). FDA interprets this to mean that the COA must have content validity, construct validity, reliability, and the ability to detect change. But COAs can do much more. For example, COAs can be included in labeling claims, as with CRYSVITA (burosumab-twza) for X-linked hypophosphatemia linked **here**, which incorporates both PRO and ClinRO measures. COAs can even lead to a regulatory change in thinking about a particular disease or condition. For example, just over two months after hearing directly from patients with epidermolysis bullosa (EB), a rare disorder that results in serious cutaneous manifestations, at an externally-led PFDD meeting, FDA published a draft guidance for sponsors developing therapies for EB that outlined specific examples of efficacy endpoints that would show the drug provides a clinically meaningful improvement. The finalized guidance can be found **here**.

If you are considering developing or utilizing in your clinical development program a COA, or if have

questions about other PFDD initiatives such as PFDD meetings, we encourage you to contact your Goodwin life sciences lawyer for assistance on how to incorporate the patient voice–the real experts on their disease or condition—in drug development.