CLINICAL TRIALS TRANSFORMATION INITIATIVE

CTTI Tool
Of Special Note With Regard To Patient-Reported Outcomes

Patient-reported outcomes (PROs) are an important part of patient-centered trial design. They allow patients to provide investigators with information that can only be assessed by the patient, such as pain, quality of life, and fatigue. They may also save time, effort, and resources. Therefore, the collection of PROs is encouraged, when appropriate. However, it is also important to be conscious of the relative burden on the patient and site staff and work to ensure the PRO measure selection and reporting frequencies are fit for purpose and that the relative burden of completion is minimized.

Unfortunately, the optimal number of PRO measures and the reporting frequencies that are minimally burdensome have not been defined, because each trial and population is unique. The goal is to find the minimal frequency that will still produce meaningful results.

When selecting PROs and the frequency of their measure, key concepts to keep in mind include:

1. What are the characteristics of the symptom being measured?
    - Does it come and go daily, or is it something that can be measured weekly or monthly?
2. What is the natural history of the disease and treatment characteristics?
    - How long before the treatment will take effect?
    - Once symptoms resolve, will they remain under control, return or worsen?
    - Is this an acute or life-threatening disease or a stable, chronic disease?

      (Stable or static diseases should use a lower frequency of assessments than more dynamic or acute diseases)
3. What are the study population characteristics?
    - Is this a healthy population or extremely ill population?
4. What is the duration of the trial?
    - A short trial with a need to assess small changes in a short period may require more frequent assessments
    - An extended trial may allow more time to be taken between assessments
5. What is the PRO's level of importance in the assessment of treatment benefit?
    - How critical the assessment is to understanding the overall treatment benefit may determine how frequently it should be measured

If appropriate, include the FDA early in discussions of data collection, procedural burden, and the use of PROs