

SHOULD I INCLUDE PATIENT REPORTED OUTCOMES (PROs) IN MY STUDY?

Collecting patient-reported outcomes (PROs) is an important part of patient-centered trial design. However, it is important to consider the relative burden on the patient and site staff, and also ensure that the PRO measure selection and reporting frequencies are fit for purpose and that the relative burden of completion is minimized.

Because each trial and population is unique, there isn't a set standard when it comes to collecting PROs—the goal is to find the minimal frequency that will still produce meaningful results. When selecting PROs and the frequency of their measure, trial designers should consider:

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, the FDA should also be included early on in discussions of data collection, procedural burden, and the use of PROs.