

Module 10 – Quality of Life Research

Special Considerations for QoL Endpoints

Quality-of-life endpoints can help researchers gather important information about how to improve the lives of people with spinal cord injury that can't be detected any other way. But these endpoints can be more complicated to develop and use, and they can take a long time to validate.

To understand why, let's compare them with the kinds of endpoints that are used in other research, such as drug and device R&D. Trials of drugs and devices usually use endpoints that can be measured without relying on opinion or interpretation. Examples include measuring changes to the spinal cord on an MRI, measuring how far someone can walk within 6 minutes, counting the number of bowel movements in a day, or using quantitative tests like blood pressure measurement.

These endpoints are often called “clinical endpoints,” or “hard outcomes,” because they rely on objective, quantitative measurements of clinical symptoms or other physical phenomena.

The advantage of objective endpoints like these is that they are relatively easy to measure, and the results are considered to be reliable and less vulnerable to bias than subjective endpoints. Together, these characteristics increase confidence that the research results are valid, or “real.”

However, a limitation of objective endpoints is that many of them give no information or only limited information about how a condition affects a person's ability to function, or the way they feel. For example, objective endpoints for some symptoms can measure whether the symptom happened or how often it happened, but they cannot measure the intensity of the symptom, or how it made someone feel.

In contrast to “clinical endpoints,” quality-of-life endpoints are, by definition, subjective: they measure how someone feels or what they think, which is subject to their opinions, interpretations, experiences, and biases. This does not mean that QoL endpoints are not valid, it just means that they have to be rigorously developed and tested to ensure that they reliably measure what they are supposed to be measuring.

Let's take pain endpoints as an example. Pain is often measured using a PRO tool called the Visual Analog Scale, or “VAS.” You may have seen or used this tool yourself at the doctor or in a hospital.

The way it looks can vary, but it usually shows a rating scale from 0 to 10, with text describing pain intensity, and it may include cartoons or emojis representing the way the pain makes you feel.

A person who is experiencing pain is asked to select a number or make a mark on the scale to show the intensity of their pain at a specific time point, for example, their current level of pain, or their level of pain over the last 24 hours, or the last week.

In routine healthcare, the person's answers are compared with previous answers to tell how much their pain is improving or worsening. The Visual Analog Scale can be used the same way in a clinical trial testing whether a pain treatment is effective.

The visual analog scale has several benefits:

- It is simple for most patients to fill out and for doctors or researchers to “score”
- Over time we have seen that its results are consistent with other ways of measuring pain, which increases confidence in its validity as a measure (although not as much as if there were objective measures the VAS could be compared to).
- Research that compares the results of the test when used repeatedly has shown that it is reliable, meaning the results are consistent.
- The visual analog scale is sensitive enough to detect changes in an individual's pain over time.

However, it also has limitations:

- It can be difficult to use for people who have dementia or cognitive impairment.
- It is less reliable in people with low literacy.
- Differences in the text used to describe pain intensity can change the way people score their pain.
- It is less useful for comparing one person's pain to another person's pain, because the way different people experience and therefore assess pain is different.

The Visual Analog Scale is a relatively simple PRO that asks only one question and uses few words. But this example shows how even a simple PRO can provide different results with different people or when there are small changes in the way it is presented. For a more complex PRO, such as a questionnaire that asks about many different aspects of quality of life, these challenges are multiplied.

Therefore, PROs need to be tested through research to ensure that they are valid and reliable, and they need to be assessed for their ability to make comparisons between different groups of people, such as a treatment group and a placebo group in a clinical trial.

Research advocates can be instrumental in helping to evaluate the utility, reliability, and validity of PROs by:

- Helping to design PROs
- Helping to design research studies to test a PRO's reliability and validity
- Advising on who from the community should be included in tests of a PRO, and
- Interpreting the results of the research testing a PRO.