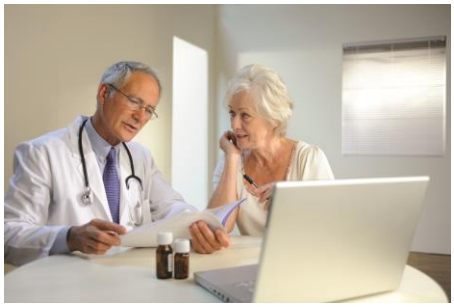
PATIENT-REPORTED OUTCOMES



A critical part of demonstrating the need, safety and effectiveness of a technology is a comprehensive assessment of outcomes that are important to patients and which guide patient management. Patient Reported Outcome (PRO) assessment is concerned with determining which outcomes are most important and how best to measure and value these outcomes to ensure valid and meaningful endpoints for decision makers. Effective PRO assessment is essential to differentiate a technology as much as possible from its alternatives.

PATH has extensive experience in analyzing early trial data and in conducting literature reviews to identify potentially suitable outcomes, measures and instruments for PRO assessment. Depending on the disease, intervention and technology, PATH uses a wide range of PRO measures including: general disease measures (e.g. morbidity, mortality, long-term disease progression/prognosis); treatment-specific measures (e.g. success rates, relative risk reductions, safety measures like side effects and adverse events, patient adherence like compliance and persistence); and preference-based measures (e.g. patient or provider satisfaction, quality of life (QOL), and discrete choice experiments for willingness-to-pay valuation).

Measures for these PROs are typically in natural units or can be measured using existing validated instruments. However, it is common that new PROs need to
be developed entirely and in this case PATH has decades of experience in:

- Health surveys (patients, providers, caregivers and general public)
- Patient and provider focus groups
- Key opinion leader (KOL) judgment and attitude assessment
- Consensus meetings and Delphi panels
- In-person, telephone, mail or self-administered interviews (paper, computer-based or web-based)
- Patient chart reviews or analyses of longitudinal databases/registries
- Analyses of linked administrative databases
- Development of disease-specific satisfaction and QOL instruments

PATH specializes in the development of QOL instruments (including reliability, validity and responsiveness testing) and in the assessment of QOL on patient and general populations. We focus on disease-specific instruments for maximum sensitivity, on generic scales or indices (e.g. SF-36) to permit comparability across disease areas, and in utility elicitation using utility-based indices (e.g. HUI, EQ-5D)
or direct patient measurement (standard gamble, time trade off) to be used in
cost-utility analyses.