

Roadmap to **PATIENT-FOCUSED OUTCOME MEASUREMENT** in Clinical Trials

Understanding the Disease or Condition **1**

A. Natural history of the disease or condition

- Onset/Duration/Resolution
- Diagnosis
- Pathophysiology
- Range of manifestations

B. Patient subpopulations

- By severity
- By onset
- By comorbidities
- By phenotype

C. Health care environment

- Treatment alternatives
- Clinical care standards
- Health care system perspective

D. Patient/caregiver perspectives

- Definition of treatment benefit
- Benefit-risk tradeoffs
- Impact of disease

Conceptualizing Treatment Benefit **2**

A. Identify concept(s) of interest (COI) for meaningful treatment benefit, i.e., How a patient:

- Survives
- Feels (e.g., symptoms)
- Functions

B. Define context of use (COU) for clinical trial:

- Disease/Condition entry criteria
- Clinical trial design
- Endpoint positioning

C. Select clinical outcome assessment (COA) type:

- Patient-Reported Outcome (PRO)
- Observer-Reported Outcome (ObsRO)
- Clinician-Reported Outcome (ClinRO)
- Performance Outcome (motor, sensory, cognition)

Selecting/Developing the Outcome Measure **3**

A. Search for existing COA measuring COI in COU:

- Measure exists
- Measure exists but needs to be modified
- No measure exists
- Measure under development

B. Begin COA development

- Document content validity (qualitative or mixed methods research)
- Evaluate cross-sectional measurement properties (reliability and construct validity)
- Create user manual
- Consider submitting to FDA for COA qualification for use in exploratory studies

C. Complete COA development:

- Document longitudinal measurement properties (construct validity, ability to detect change)
- Document guidelines for interpretation of treatment benefit and relationship to claim
- Update user manual
- Submit to FDA for COA qualification as effectiveness endpoint to support claims

