

AGENDA

- Why PROs ?
- Applications of PROs in Drug Development
- US Healthcare Reform Enhanced Value of PROs
- Key Takeaways

Patient Perspective is Increasingly Valued....and Expected!

Importance of including the patient's perspective in drug development is increasingly recognized

Significance of including the patient's perspective has risen from multiple perspectives

Guidance: EMEA Reflection Paper on Health Related Quality of Life (2005); FDA PRO Guidance (2009); FDA Draft Guidance for Industry: Qualification Process for Drug Development Tools (2010); EMA DDT in draft (2012)

Critical-Path Institute's PRO Consortium (public/private partnership) developing new PRO tools (formed 2008) for use as endpoints making regulatory claims

Patient Centered Outcomes Research Initiative (PCORI)— expects patient involvement in research, and meaningful outcomes to be measured and communicated in meaningful way — to the patient National Quality Forum — focusing on how to apply PROs to performance measures, for potential evaluation of providers, etc.

PDUFA FDA Patient Focused Drug Development Initiative – actively incorporating patient perspective into what diseases should be of focus

Health Care Decision Makers – using PROs in interventional /disease management programs as well as tools used to assess patient outcomes at the point of care. Providers are also recognizing incorporation of Patient Perspective as a **source of competitive advantage**



FDA Roadmap to Patient-Focused Outcomes Measurement

Roadmap to PATIENT-FOCUSED OUTCOME MEASUREMENT in Clinical Trials

Understanding the Disease or Condition

Conceptualizing Treatment Benefit

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Selecting/Developing the Outcome Measure

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- 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

- 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)

- 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



U.S. Food and Drug Administration Center for Drug Evaluation and Research Office of New Drugs http://www.fda.gov/Drugs

Application of PROs in Drug Development

CONCEPT ELICITATION

- Disease and treatment impact
- Definition of treatment benefit
- Benefit-risk tradeoffs

EXIT INTERVIEWS

- Explore indications
- •Understand benefits vs. risks
- •Substantiate or complement other PRO measures
- •Highlight potential issues for adherence to treatment
- •Identify subpopulations with greatest response or patients unlikely to benefit from the treatment

PATIENT PREFERENCE-UTILITIES

- •Complementary to clinical and safety data on benefits and risks of new treatments
- •Used in economic evaluation to inform resource allocation decisions

CONJOINT ANALYSIS

- Used to understand patient preferences
- Benefit-risk tradeoffs
- Inform drug development decision-making

Why Should PROs be Important to HTAs/ Payers?

- ■Basis for indication primary or co-primary endpoint in certain symptomatic diseases
- Differentiate and add value (e.g. key secondary endpoint in labeling)
- Demonstrate meaning of primary endpoint
- Complementary information, to support treatment benefits even if not in the label (e.g. publication, global value dossiers, HTA submissions)

US Healthcare Reform: Enhanced Value of PROs

Changes in US Healthcare (HC) system have lead to <u>transformation in delivery systems</u> with focus on:

- Decreasing rising HC costs
- Increasing patient satisfaction
- Improving patient outcomes

Implication of these changes:

- Need for simple, easy to use patient tools -
 - Disease awareness
 - Clinical practice tools that can be administered at the point of care

Challenges:

- Minimal evidence demonstrating improved patient outcomes results in reduction in HC costs
- Incorporation into EHRs

Opportunities and Challenges Future Trends will Continue to Enhance the Value of PROs







TECHNOLOGY:

Mobile Computing Smartphones/Tablets Apps Social Media mHealth eHealth Records Big Data Analytics

CLINICAL TRIALS:

Sensor technologies Real-time tracking Remote labs Drug adherence Patient selection/retention Personalised medicine The Digital Pill

HEALTHCARE:

Economics of healthcare Pharma industry consolidation R&D attrition and productivity Virtual HCPs Local clinics (non-MD) Developing economies

Key Takeaways



- PROs can show important differences in efficacy/tolerability to support access for medicines
 - **Likely to be increasingly important in the future** PROs in drug development, disease awareness tools, clinical practice tools

Implications for pharmaceutical industry

- Develop a PRO strategy early and proactively
- Closely review in which TAs PROs make a difference
- Monitor what and how PROs are being used (e.g. as performance measures, clinical practice tools, labeling claims, etc)



Thank you for your attention!

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