Incorporating Patient Reported Outcomes (PROs) in NCI- sponsored Clinical Trials (U10s)

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Issues

- **Multiple Different NCI & NIH PRO Initiatives & Activities**
  - Each with Different Purposes
  - Each In Various Stages of Development
  - Each Requires Different Expertise
- **Need for Clarity in the Incorporation of PROs/HRQOL into NCI-sponsored Clinical Trials**

Key Initiatives & Activities

- **PRO Endpoints in NCI Clinical Trials**
  - Secondary Endpoints in Treatment Trials (PRO & HRQOL)
  - Primary Endpoints in Symptom Management Trials
- **Curation of HRQOL Tools for caDSR**
  - Common Data Elements
  - Different Approach for HRQOL (whole instrument)
  - Integration for Medidata Rave
- **PRO-CTCAE**
  - Symptomatic Toxicity Measurement System
- **PRO Core Domains**
  - Collection of Common PRO Domains Across Clinical Trials
  - Three Disease Specific Domains

PRO Endpoints in NCI Clinical Trials

- **Incorporate PROs into NCTN/NCORP Clinical Trials**
  - NCI Ensure the Hypothesis-driven Inclusion of PROs
    - Clinical Context, PRO Expertise, Statistical Analysis
    - Review Rationale for Inclusion and Analysis
    - Treatment Trials Different Issues than Symptom Management
- **Community Needs Clarity**
  - PROs for Symptoms, Toxicities, Functional Assessments & HRQOL
- **Framework Needed**
  - Overall Concept for Inclusion that Does Not Dictate, but Provides Guidance to Investigators, Reviewers on Use

Curation of HRQOL/PROs for caDSR

- **Users Put PRO Content into caDSR**
  - Often Multiple Data Elements Support One Measure
  - Tools Difficult to Find by Other Users
- **Numerous HRQOL/PRO Measures now in caDSR**
  - 30% PRO Content Curated Based Upon Best Practices
  - 70% PRO Content not Curated with Best Practices
  - Need Users to Review and Retire Redundant PRO Content
- **Common Data Elements Curation of HRQOL & PROs Started**
  - HRQOL Project Plan Developed
  - Call for Membership for HRQOL Curation Working Group
  - Facilitate Integration with Medidata Rave

PRO-CTCAE

- **Sandra Mitchell, PhD to Present**
- **Measurement System for Capturing Real Time Patient Reports of Symptomatic Toxicities**

Core Set of PRO Domains For Trials

- **Consensus Development of PRO Core Domains**
  - Common, Consistent, Clinically Relevant Symptoms Across Cancer Sites
  - Use Across Studies to Facilitate Treatment Effect & Cross Trial Comparison
- **Disease Specific Domains**
  - Ovarian Cancer, Head & Neck Cancer, Prostate Cancer
  - Multi-Modality Therapy with Symptomatic Toxicities
- **Presented March 2013 CTAC**

Existing Working Group & Committee

- **SxQOL Steering Committee**
  - Review of Symptom Management Trials
  - Liaisons to Disease Steering Committees for PRO & HRQOL Review on Treatment Trials

- **NIH/FDA Outcomes Assessment Working Group**
  - Coordination Activities Between NIH ICs & FDA
  - Development of Tools for Outcomes in Clinical Trials
  - Patient Reported, Clinician Reported, Observer Reported

New Coordination Activities (To Be Formed)

- **Internal NCI Patient Reported Outcomes Working Group For NCTN/NCORP Clinical Trials**
  - Coordinate & Formalize the Internal NCI ad hoc Discussions
  - Build on Success of Coordination of PRO-CTCAE

- **New Working Group with External PRO Investigators & NCI**
  - Develop Framework for Inclusion of Different PRO Assessments Across NCTN/NCORP Clinical Trials.
  - Short-term (12-18 months)
    - Primarily through Conference Calls, In-person Meeting
    - Membership from QOL Experts in Groups, SxQOL, Liaisons to Disease SCs

- Questions?
- Discussion