Genesis of the PRO Consortium & Benefits of Collaboration

Wendy R. Sanhai, Ph.D., M.B.A.
Office of the Commissioner, FDA

FIRST ANNUAL PATIENT-REPORTED OUTCOMES (PRO) CONSORTIUM WORKSHOP

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Initial Governance/Activities under PRO Consortium

• Agreement: between C-Path and members
• FDA is not a signatory
• FDA is a partner:
  • No solicitation of funds
  • Independent review of dossiers
  • Business decisions among C-Path and members are outside of FDA domain
PRO Qualification Process - Evolving

- Consortium designs (scoping documents) and conducts studies for development of instrument & supporting data for dossier (development stage)

- FDA provides input at key milestones and critical decision points

- Dossier submitted to FDA review, with request for formal Guidance, and with summary placed in public domain by Consortium

- FDA acknowledges receipt of dossier and later issues FR notice of intent to develop Level 1 Guidance

- Formal FDA review, and development of draft Guidance as deemed appropriate

- Issue of draft Guidance
Consortium Develops PRO Instrument Dossier to FDA

Informal Review by FDA Review Team

Request Additional Information

Consortium presents Dossier & Formal request for Guidance to FDA

FDA begins formal review & issues FR notice of intent to develop Guidance

FDA Issues draft Guidance

Information in public domain
Why PRO Consortium?

• Efficiency for industry/FDA: time and resources

• Standard approaches for submissions to support *(content i.e. questions)* validation and qualification *(fit for purpose...measures that support labeling claims)*

• Timely delivery of PRO instruments to patients and clinicians---and FDA
Collaboration Model

Stakeholders
- Pharmaceutical Companies
- Medical Device Companies
- Professional Organizations
- Patient Advocacy
- Academia
- Others

C-Path (Neutral Ground Administrative Coordinator)

PRO Consortium

FDA (Federal Liaisons)

Other Federal Agencies: NIH, AHRQ, (PROMIS), CMS

Coordinating Committee

Work Group 1
- Instrument A

Work Group 2
- Instrument B

Work Group 3
- Instrument C

Work Group 4
- Instrument D

Process Work Group

Review/Feedback From Partners/FDA/Others

PRO Instruments Fit for Use
Data Shared in the public domain
PRO Consortium Progress

• 23 member firms
• 6 disease workgroups
  – Asthma
  – Advanced Breast Cancer
  – Cognition
  – Depression
  – IBS
  – Non-Small Cell Lung Cancer
• Process workgroup
  – Staging for instrument development
  – Scoping phase summary document template for FDA submission
  – Vendor selection process (template RFP & selection criteria)
  – New workgroup proposal process
  – Guidelines for non-member participation
Benefits of This Collaboration

- Collaboration allows FDA to work with multiple partners to leverage expertise and resources toward PRO instrument development
  - Improved/transparent FDA internal (review) processes
  - Pooling of industry know-how and resources
  - Provide a basis for eventual comparison of labeling claims by physicians
  - Issuance of best practices and Guidances toward future instruments and product development
Benefits to Patients

• Content validity: evidence from patients in target population, on functionality, symptoms and disease progression...empowering patients
  – Later correlate with physician global

• Input in development of metrics to evaluate their own health status
  – understand questions/give the correct answer
  – Valid measure of a set of symptoms...correlate with outcome measures in clinical studies
Benefits to Partners

• Industry: transparency in PRO Instrument development
  – Input into concepts: test formats, methodology, nomenclature, scoring algorithm/s

• Informed product development: a well-developed PRO, developed with patient input, is enormously helpful in large trials

• Potential for applying qualified PRO instruments in multiple drug development programs
Benefits to FDA

• Build on PRO Guidance (December, 2009)

• Learn with the development of each PRO Instrument developed

• Consistency of submission...easy during review/endpoint assessment

• Inform the development of future instruments & Guidances

• Harmonization with international regulators: EMA
PPP Value Assessment

• FDA partnering with U Maryland to develop metrics to assess performance and outcomes PPP programs...value to stakeholders and patients

• PRO consortium will be evaluated as case study: What’s working/what’s not, how can we improve

• Ritu Agarwal & Kenyon Crowley, Director and Associate Director, Center for Healthcare Information and Decision Systems
Balanced Scorecard

Mission: Improve Public Health Through Scientific Discovery

- Production of public-domain scientific knowledge
- Research outputs
- Increase in partners’ knowledge and skills
- Development of new capabilities
- New knowledge creation mechanisms

- Reduced time to market
- Reduced development costs
- Reputational gains, visibility, networking
- Financial viability/ resource adequacy

Scorecard for an FDA PPP

- Right partners at the table
- Open and trusting environment
- Engagement and contributions by all partners
- Efficient and effective governance
- Documented and repeatable processes

- Public
  - Assurances of safety, security, and efficacy
  - Innovative drugs, devices, and therapies
  - Improvements in understanding
- Partners
  - Varied returns
  - Satisfaction