

Patient Reported Outcomes (PRO) Consortium

Progress so Far and What's Ahead... **2009 Annual DIA Meeting, San-Diego**

Priti Jhingran, Ph.D.
GlaxoSmithKline

PRO Consortium: Coordinating Committee Kick-Off Meeting

- Date/Location: March 19th, 2009 – Washington DC.
- Attendees: FDA Representative, Members, C-Path Institute
- Agreement on the Overall Goals PRO Consortium:
 - Devise an iterative, transparent, efficient process to develop new standardized PRO tools in a collaborative, pre-competitive space.
- Proposed actions for this meeting:
 - Agree on the scope
 - Propose a clear process for identifying, prioritizing & developing PRO instruments
 - Propose process for establishing & implementing working groups
 - Preliminary planning of consortium management & operations
 - Decide on next steps

Presentation Overview

- Scoping
- Prioritization
- Framework/Roles/Process
- Progress so far

Scoping and Prioritization

PRO Consortium: Scope: New PROs

- Diseases where a primary PRO endpoint is needed and where one does not exist now (e.g., symptoms, signs, functions)
- Symptoms related to multiple diseases (e.g., cognition, fatigue, insomnia, etc)

Note: PROs for use in regulatory decision-making and to support labeling claims

PRO Consortium

Scope: Existing PROs

- Historical PROs developed and implemented prior to the PRO Graft Guidance Document where:
 - Development may not be based on patient input
 - May have potentially 'dubious' developmental heritage
 - May be in need of further qualitative and quantitative validation

PRO Consortium: Selection of PROs

- Criteria of selection of PROs in specific research areas should be transparent and neutral. Some key criteria include:
 - Disease area with unmet need
 - Product development area of interest to multiple sponsors
 - Disease area where the patient experience is important
 - Disease area with regulatory 'demand' for a pre-competitive PROs based on feedback from FDA/SEALD
 - Diseases currently reliant on more 'objective' measurement where subjective impact of disease via PRO assessment should be explored to expedite drug development

PRO Consortium: Selection of PROs – Other important Criteria

- Contributing members willing to allocate time of relevant in-house experts and/or provide start-up funds.
- Companies have collaborative ideas to propose.
- Probability of funding a PRO project is high

PRO Consortium:

Potential Projects - Diseases

Acne symptoms and signs

Acute exacerbations of COPD

Acute otitis externa

Acute otitis media

Anemia

Asthma

Cachexia S/S

Chronic Idiopathic constipation

Chronic Sinusitis

Chron's Dz S/S

Depression

Diabetes

Epilepsy

Fatigue related to HCV

Fatigue related to COPD

Schizophrenia (Functional Status)

Pain (symptoms and impact)

Dyspepsia

Dyspnea

GE Reflux

IBS

Insomnia

MCI

Male Hypogonadism

Menopause

Neuropathy

Oncology

general, lung, renal, prostate
brain, breast, colorectal, NHL

Osteoarthritis

Obesity

Overactive Bladder

Psoriasis

Rheumatoid Arthritis

Ulcerative Colitis

Note: Red indicates highest priorities based on member voting. Blue indicates 2nd level.

PRO Consortium: Potential Projects: Symptoms Related to Multiple Diseases

- Pain
- Cognition
- Fatigue
- Insomnia
- Cachexia
- Neuropathy
- Dyspnea
- Activities of Daily Living

Note: Red indicates highest priorities based on member voting. Blue indicates 2nd level.

PRO Consortium: Establishment of Scoping Groups

GOAL: To define the scope of activities for the Consortium.

- **Identify areas of unmet need (must do)**
- **Develop draft claims (must do)**
- Proposed conceptual framework of instrument (e.g. symptoms, impact of symptoms)—
what is the general strategy?
- Review of approvals in that area
- What is currently ongoing in the area? Low-hanging fruit? Are there tools in
development?
- Participation from other potential partners
- Any interaction with Review Divisions or other regulatory history? (e.g. guidance, etc.)
- Severity of need
- What are the standard endpoint models? What are the other clinical endpoints?
- Population targeted
- Timeline for development
- Leadership/participation

Note: These may or may not lead to the establishment of Working Groups.

Framework/Roles/Process

PRO Consortium: Framework



PRO Consortium:

Roles: Preliminary Discussions

- **C-Path: Director;**
 - Convene meetings, coordinate work of the Coordinating Committee and working groups, and facilitate communication among members.
- **Industry:**
 - Representatives will participate in consortium as described in Agreement.
 - Companies will collaborate with each other and with FDA to develop standardized PRO instruments.
 - Appoint a Co-Director to serve as representative/voice of industry members.
- **FDA**
 - Guide, advise the consortium.
 - Serve as federal liaison; no fiduciary role or voting rights.
 - Interact with consortium as a whole.
 - Ensure Reviewing Division engagement and alignment (including SEALD).
 - No solicitation of funds
 - No independent reviews
 - Dossier goes to FDA review team
 - Conduct a review process to determine *fit for purpose/qualification*

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Process Working Group

■ Key areas of focus

- What are the Co-coordinating Committee's responsibilities? What should be delegated to subcommittees (Scoping and Working Groups)?
- What is the flow chart for decision points for the PRO development from Scoping Group through submission to FDA?
- What are the milestones within PRO development process where a FDA consult will be obtained ?
- How do we formalize a selection process?
- How do we share information publicly?
- How do we manage involvement of non-consortium members?
- Can do we leverage PROs already in development ?

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STAGING FOR INSTRUMENT DEVELOPMENT

- **Pre Stage I-** identify PRO Instruments
- **Stage I-** in depth lit review, work group formation, gather expert opinion, develop protocol for qualitative research
- **Stage II—** Perform qualitative research—include patients from target population, develop first draft of the PRO Instrument
- **Stage III-Refine** the PRO Instrument, assess measurement characteristics of instrument
- **Stage IV—Confirm** measurement properties of the revised instrument in longitudinal studies, assess ability of PRO instrument to detect change and develop a definition for responder to treatment

Progress So Far....

IBS Scoping Group Update

- The IBS Scoping Group is now a Working Group to develop PRO Measures for IBS-C and IBS-D
- Non-voting members in this group are from FDA (SEALD), FDA (Reviewing Division), academia and a patient advocacy group (IFFGD).
- Goal is to expedite and complete the development of these measures by the end of 2009.

Asthma Scoping Group Update

- Drafted mission statement/objectives
- Identified all PROs/claims included in labels (indication or clinical studies section) before and after 2006.
- Currently, information on the development and validation of PRO measures being compiled
- Next steps: Discussions with FDA; pulling together documents to support discussions

Oncology Scoping Group Update

- Drafted mission statement/objectives
- Identified tumors/priorities, based on cancers that are prevalent and companies interest along with potential claims:
 - Top 3: Lung (non-small cell, advanced), Breast (adjuvant and advanced), Brain (advanced)
 - Other 3 tumor types: Colorectal (adjuvant and advanced), Prostrate (advanced), Ovarian (advanced)
 - Identified multiple symptoms across tumor types (e.g. pain, fatigue)
- Identifying existing PRO Groups working in Oncology
- Next steps: Discussions with FDA and establishment of Working Groups

Diabetes/Depression Scoping Group Update

- Currently in the process of drafting the mission statement
- Determine PRO measures in diabetes/depression that are acceptable to FDA
- Identifying measures currently under development
- Identifying unmet needs for PRO Measures.

Key Takeaways

- Progress so far is commendable.
 - Many thanks to contributing members/experts who have allocated time and effort
- Having C-Path provides a great momentum to push things along.
- FDA's fully commitment to ensure the success of the initiative.

Thank you for your attention!

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**Priti Jhingran
GlaxoSmithkline
priti.m.jhingran@gsk.com**