Second Annual Patient-Reported Outcome (PRO) Consortium Workshop

March 15, 2011 ■ Silver Spring, MD

Co-sponsored by

Patient-Reported Outcome (PRO) Consortium: Update

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Goals of PRO Consortium

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- Enable pre-competitive collaboration that includes FDA input/expertise
- Avoid development of multiple PRO instruments for same purpose
- Share costs of developing new PRO instruments
- Develop qualified, publicly available PRO instruments
- Facilitate FDA's review of medical products by standardizing PRO endpoints

Membership

- **Voting Members**

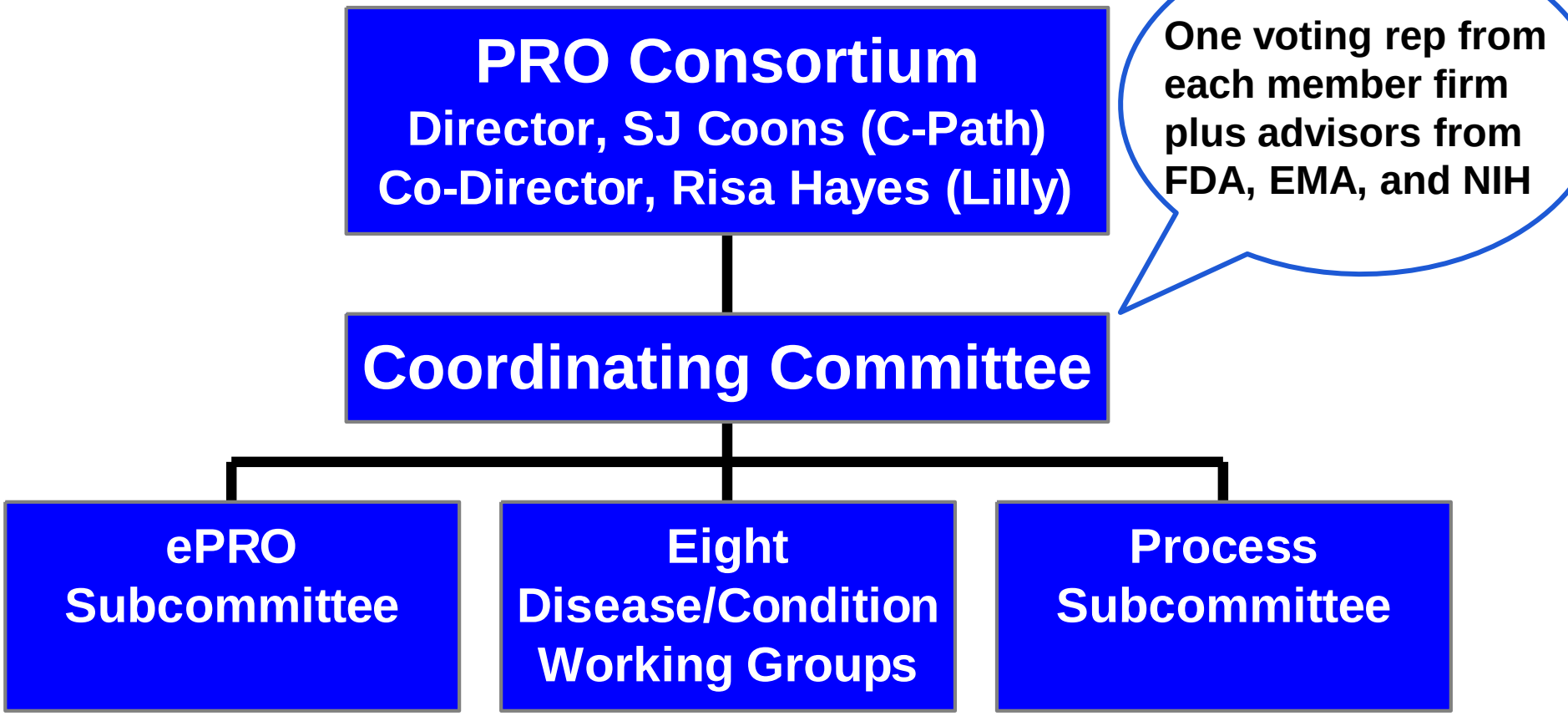
  Medical product (pharmaceutical, diagnostic, and medical device) companies

- **Non-Voting Participants**
  - Liaisons/advisors from governmental agencies
  - Clinician consultants, patient advocates, academic researchers, and commercial entities partnering in the development of the PRO instruments

Members

- Abbott
- Actelion Pharmaceuticals
- Allergan
- Amgen
- Astellas Pharma
- AstraZeneca
- Boehringer Ingelheim
- Bristol-Myers Squibb
- Daiichi Sankyo
- Eisai
- Eli Lilly & Company
- Forest Laboratories
- GlaxoSmithKline
- Ironwood Pharmaceuticals
- Johnson & Johnson
- Merck Sharp & Dohme Corp.
- Novartis
- Novo Nordisk
- Pfizer
- Roche
- sanofi-aventis
- Shire
- Sunovion
- Takeda Pharmaceuticals
- UCB

Organizational Chart



Over 150 scientists and/or clinicians participate

Working Groups as of Last Year's Workshop

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- Asthma
- Breast Cancer
- Cognition (Mild Cognitive Impairment)
- Depression
- Irritable Bowel Syndrome
- Lung Cancer (NSCLC)

New Working Groups

- In February 2010, proposals for the creation of seven new working groups were submitted by member firms for consideration
- FDA reviewed six submitted *Feasibility Documents* based on the proposals
- FDA was willing to consider PRO instruments for four of the six proposed new areas
- Two new working groups were authorized at the end of 2010
  - Functional Dyspepsia
  - Rheumatoid Arthritis

Path to Qualification of a New PRO Instrument

- *Feasibility Document*
- *Scoping Stage Summary Document*
  - Proposed target population, concepts, conceptual framework, labeling language, and endpoint model (showing endpoint hierarchy)
- *Qualitative Research Summary Document*:
  - Evidence that supports the content validity of draft PRO measure, including confirmation or revision of the proposed conceptual framework
- *Quantitative Research Summary Document*:
  - Evidence supporting other measurement properties (e.g., reliability, construct validity, responsiveness) of final PRO instrument, along with user manual, and other documentation
- *Qualification Dossier*

Overall Working Group Status

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On Hold

- Breast Cancer WG

Scoping Stage

- Functional Dyspepsia WG
- Rheumatoid Arthritis WG
- Lung Cancer WG

Vendor Selection Stage (prior to qualitative research)

- Asthma WG
- Depression WG

Qualitative Research Stage

- Irritable Bowel Syndrome WG
- Cognition WG