SECOND ANNUAL PATIENT-REPORTED OUTCOME (PRO) CONSORTIUM WORKSHOP

March 15, 2011 ■ Silver Spring, MD

Co-sponsored by

FDA/NIH Interagency Outcomes Assessment Working Group: Goals & Updates

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FDA-NIH Collaboration

- February 24, 2010, FDA & NIH announced the formation of a joint leadership council for Advancing Regulatory Science through Novel Research and Science-Based Technologies

- October 25, 2010 – FDA & NIH representatives meet for first meeting of the Interagency Outcomes Assessment Working Group (IOAWG)

Catalyst For IOAWG

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- Improve interagency communication on outcomes assessment development and qualification

- Identify and develop opportunities for agency staff and other scientists developing outcomes assessments to:
  - Increase training and education/outreach
  - Inform the use of scarce resources and staff-time toward direct patient benefit
  - Indentify opportunities for joint activities re: data acquisition, annotation and management to develop standards, best practices and inform clinical and regulatory decision-making
  - Increase transparency of respective activities

- Provide transparency and regulatory assistance related to the qualification process…as outlined in the draft DDT guidance

Clinical Outcomes Assessments

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Definition Includes:

- Patient-Reported Outcomes
- Clinician-Reported Outcomes
- Observer-Reported Outcomes

NOT "Biomarkers"

Objectives of IOAWG

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- To increase communication between FDA and the many separate efforts at NIH to advance measurement science and knowledge base for improving clinical outcomes
- The help the parties understand and inform the regulatory processes for evaluation of the clinical outcomes assessments
- To foster appropriate evidence generation toward the qualification of novel clinical outcomes assessments
- To leverage public and private efforts toward consensus and standards development in this area

IOAWG Membership

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- FDA:
  - Office of the Commissioner
  - Center for Drug Evaluation and Research
  - Center for Devices and Radiological Health
  - Center for Biologics Evaluation and Research
- NIH
  - Office of the Director
  - 6 ICs: NCI, NIA, NIAMS, NICHD, NINDS, NHLBI (perhaps more…)
- Additional representation as needed

Updates…

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- Established regular meetings (3 meetings to date)
- Drafted work group charter and memo to inform FDA/NIH Leadership Council
- Drafted overall objectives
  - Described qualification process for outcomes
  - Developing categories of clinical outcomes assessment used in research: Definitions, dimensions end evidence needed for intended use
- Identified a list of preliminary projects/workstreams
  - Project Inventory (being developed)
  - Education & Outreach priorities (will be developed)

Project Inventory: Goals

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- Developing comprehensive inventory of activities related to development of OA instruments at NIH & FDA:
  - Identify scientific/unmet public health need
  - Evaluate on-going activities for its readiness to enter into the qualification process
  - Identify efforts that may not realize overarching agency-goals and modify accordingly

Education and Outreach

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- Identify opportunities for educating scientific community on development, clinical qualification, and use of OA tools:
  - Workshops
  - Web pages
  - Scientific publications
  - Foster collaboration with multiple stakeholders and sharing outcomes early and openly

Summary

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- FDA & NIH have identified the benefits of working together to advance the development and qualification of outcomes assessment tools toward patient benefit and to inform product development

- NIH-developed products will be entered into DDT qualification process, when they are ready

- FDA & NIH will assure that all outcomes of this collaboration are accessible by all