Advancing Health Outcomes Measurement

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- U.S. Federal Agency Activity (actual, potential)
- Perspectives on Building, Testing, and Improving an Item Bank for Chronic Disease Patient-Reported Outcomes (PROs)
- Some Enduring Challenges in Understanding and Applying PROs

Trans-NIH RoadMap Initiative:

DYNAMIC ASSESSMENT OF PATIENT-REPORTED CHRONIC DISEASE OUTCOMES

- $25 Million, 5-Year Cooperative Agreement between NIH and Investigators.
- Goal: Improve assessment of self-reported symptoms and other domains of health-related quality of life across a wide range of chronic diseases.
- Create a publicly available, adaptable and sustainable Internet-based system, the *Patient-Reported Outcomes Measurement Information System* (PROMIS) -- that will:
  - Administer CAT-based assessments
  - Collect PRO data
  - Provide instant reports to patient, providers, and researchers.
- Lay groundwork for public-private partnership to extend the PROMIS beyond its five-year development stage

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Additional DHHS Activities

- Studies involving IRT modeling, DIF testing, item banking, and CAT development are being supported by many of the DHHS agencies, institutes, and centers, including:
  - National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)
  - National Institute for Child Health and Human Development (NICHD)
  - National Institute of Neurological Disorders and Stroke (NINDS)
  - National Institute of Mental Health (NIMH)
  - National Institute on Aging (NIA)
  - National Cancer Institute (NCI)
  - Agency for Healthcare Research and Quality (AHRQ)

NCI Small Business Innovation Research (SBIR) Contract Project
Developing IRT Software for Health Outcomes and Behavioral Science Measurement

- To develop and/or adapt software that employs both traditional and modern measurement methods to respond to the needs of health outcomes, health surveillance, and behavioral science researchers.
- Key features:
  - User-friendly
    - Both the software and supporting literature needs to be translated into terms that are palatable to researchers using appropriate examples from this field.
    - Full use of the features provided by modern computer operating systems including instant on-line help.
    - Sophisticated graphics capabilities.
  - Flexible
    - Models a variety of IRT models including:
      - Dichotomous and polytomous models
      - Multi-parameter models.
    - Provides multiple fit indices.
  - Integrated modules for
    - DIF assessment
    - Linking
    - CAT

What Else Can the Federal Sector Do To Improve PRO Measurement?

- Issue additional PAs, RFAs, and RFPs (for directed methodological and applied studies)

- Support investigator-initiated projects

- Provide scientific consultation to public and private efforts to develop and use PROs

- Support additional conferences, workshops, or consensus development activities

Building, Testing, Improving an Item Bank for Chronic Disease PROs

To "keep the PROMIS" and achieve the potential benefits, what must be accomplished?

- After you build it, test it.

  - Validation

  - Head-to-head comparison studies

- Maintain, improve item bank over time

  - New items

  - Consider "branch banks" for specific diseases (?)

  - Encourage methods studies, e.g., multidimensionality issues, preference vs. non-preference-based measures, "expansive" efforts at construct validation

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Building, Testing, Improving an Item Bank for Chronic Disease PROs

To "keep the PROMIS" and achieve the potential benefits, what must be accomplished?

- Don't stifle start-up efforts to build "competing" PRO banks

- Ongoing public and private support may be required for national item bank for CAT -- at least until adequate field testing completed

- All the while, encourage a range of item bank / CAT applications to PROs -- not only in clinical research, but patient-provider decision making

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Building, Testing, Improving an Item Bank for Chronic Disease PROs

To achieve these potential benefits, what must happen?

- Adequate funding (public, private, or both)

- Regulatory agencies and other decision makers that face a choice about whether to accept CAT-generated PROs should generate clearly articulated decision criteria

- Incentive structure must encourage strong, diverse contributions from investigator community: need top-flight researchers and candidate items for bank

Building, Testing, Improving an Item Bank for Chronic Disease PROs

To achieve these potential benefits, what must happen?

- Federal research review groups (including study sections) should take balanced approach: dispassionate assessment of IRT-based applications including item bank/PROs, while maintaining flexibility to accept high-quality CTT and other "traditional" approaches.

Some Enduring Challenges in Understanding and Applying PROs

- Value-added of PROs beyond traditional biomedical endpoints ***for decision making***

- Continued probing of the "minimally important difference" (MID) in PROs -- investigating if IRT facilitates (or complicates) the issue.

- Construct validation of PROs (in the spirit of anchor-based approaches to MID) employing range of measures: utility-based, WTP, as well as biomedical outcomes

- IRT's (and CTT's) unidimensionality assumption, the multidimensionality of many PROs, and the oft noted desire for multidimensional summary score: a dilemma?

- Selecting PROs that successfully negotiate the balance between adequate ***sensitivity*** for the study at hand and ***comparability*** across studies.

- Need more longitudinal analyses of PROs (e.g., response shift)

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The Opportunity
And
The Commitment

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