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Patient-Reported Outcomes Assessment In Cancer Trials: Evaluating and Enhancing the Payoff to Decision Making

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U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES
National Institutes of Health

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Outcomes Research at NCI

- 1980s and early 1990s
  - Incremental gains in survival from some clinical trials, efforts by the QOL research and advocacy community stimulated a call for evaluation of outcomes beyond tumor response
  - NCI sponsored the Prostate Cancer Outcomes Study
    - First NCI study to examine HRQOL in a large population sample
  - FDA consideration about incorporation of HRQOL within cancer trials
- Mid to Late 1990s
  - DCTD considered establishing a special unit
  - Requested that DCCPS establish within the Applied Research Program
- Late 1990s and early 2000's
  - Established the Outcomes Research Branch
  - Ongoing NCI/FDA discussions within the FDA HRQOL Harmonization Committee

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Outcomes Research Branch

- Created in 1999 to accelerate application of new methods and technologies for measuring cancer outcomes.
- Focus on enhancing the application of “non-traditional” endpoints
  - Patient-reported outcomes a high priority
  - Across a range of applications: surveillance, research, decision making.
- Application of PRO in cancer trials
  - An initial and ongoing need

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Cancer Outcomes Measurement Working Group (COMWG)

- In 2001, convened 35 experts from academia, government, and industry.
- Assess the state of the science of outcomes measurement and identify priorities for future research and practice.
- Focused on patient-reported outcomes (PROs): Health-related quality of life, satisfaction with care/ patient needs, economic burden
- *Outcomes Assessment in Cancer: Measures, Methods, and Applications* (eds. Lipscomb J, Gotay CC, Snyder C, Cambridge University Press, 2005)

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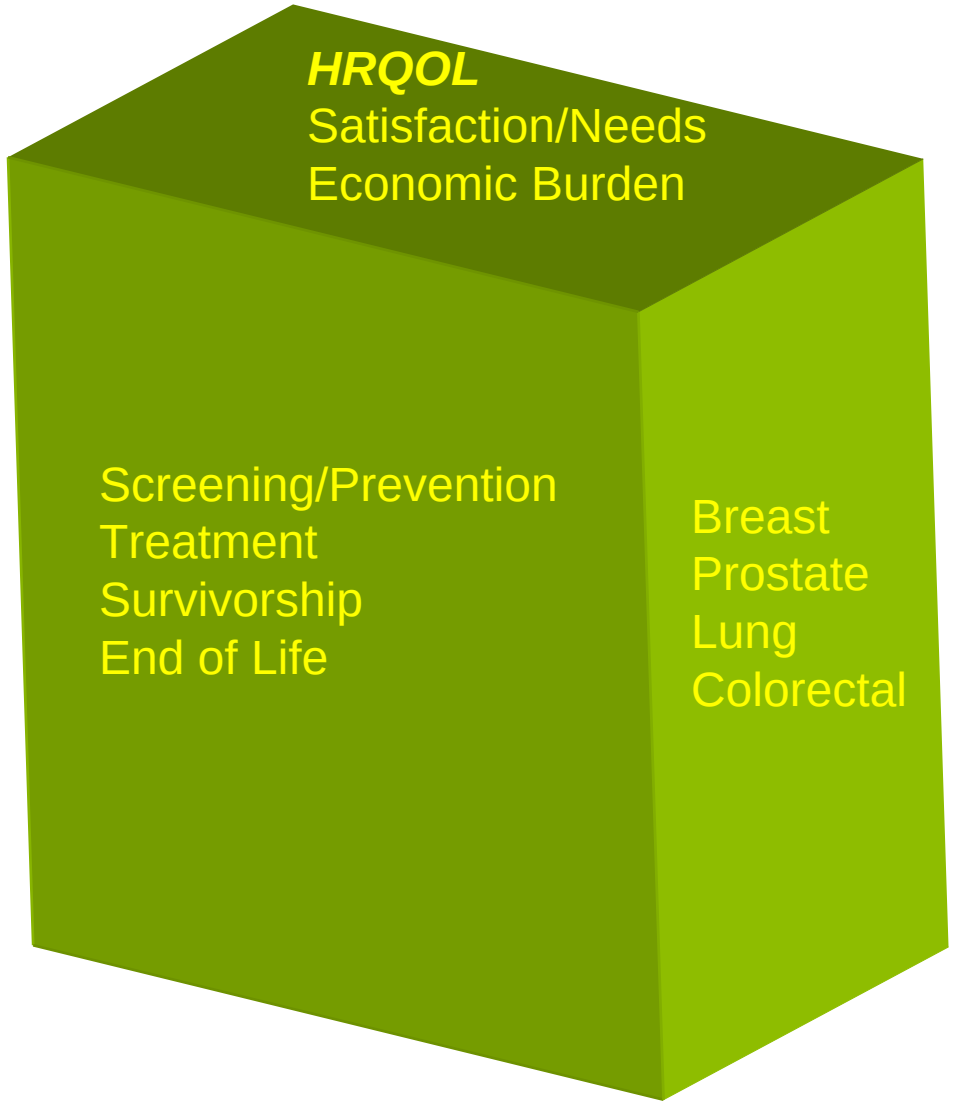
COMWG Research Focus



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Key Research Topics for PRO Measures in Cancer Trials

- Understanding and enhancing the value of PRO measures to inform decision making
- Improving the planning and execution of PRO data collection in cancer trials – missing data a common problem
- Selecting PRO measures that address the needs of specific trials and promote comparison across trials
- Exploiting modern psychometric methods (e.g., item response theory (IRT)) and applications (item banking) to improve validity, reliability, and efficiency in PRO measurement and data collection

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Patient-Reported Outcomes Measurement Information System

- **Goal:** Improve assessment of self-reported symptoms and other health-related quality of life domains across many chronic diseases
  - Core domains include pain, fatigue, emotional distress, physical function, social role participation, and overall general health.

- **NIH Roadmap Initiative:** *Re-engineering the Clinical Research Enterprise*

- **Structure:** 5-Year cooperative agreement (began Aug. 2004) between NIH and extramural investigators
  - Statistical coordinating center
  - 6 primary research and data collection sites

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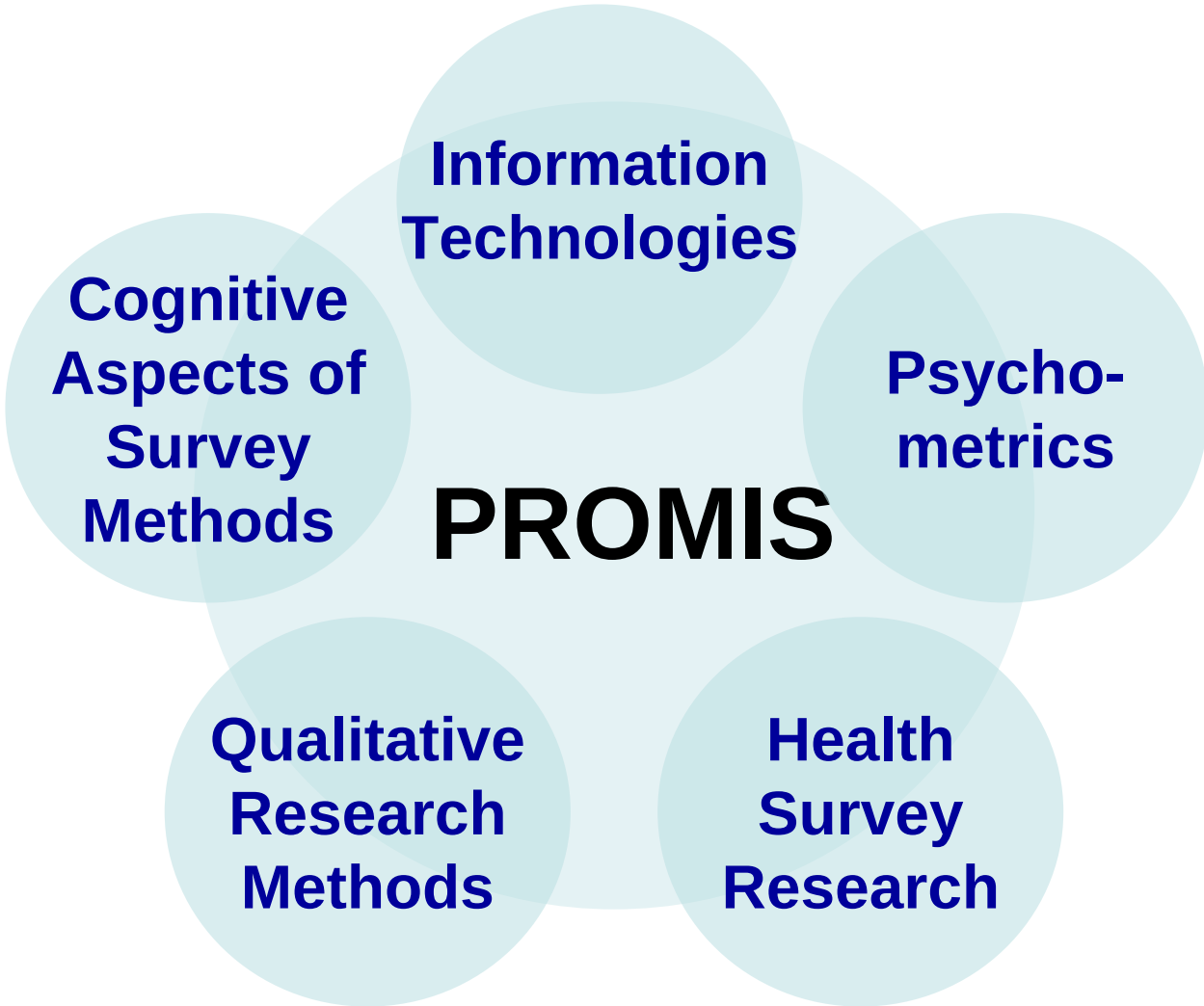
Development of the PROMIS integrates the fields of…



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PROMIS: Developing Instruments for use in Clinical Trials

- Publicly available, adaptable and sustainable Internet-based system that will provide:
  - Short Form Instruments
  - Individually “tailored” questionnaires (i.e., Computerized-Adaptive Testing)

- Advantages:
  - Reduce response burden
  - Increase scale reliability to detect group differences
  - Compare or combine results from multiple studies
  - Standardized measures within trials will enhance ability to evaluate the effectiveness across clinical trials.

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Objective of PROMIS NCI Grant Supplements

Leverage the existing PROMIS infrastructure and funding to ensure that PROMIS generates high-quality measures of patient-reported outcomes relevant to and validated for patients with cancer across the continuum of care.

- Duke Clinical Research Institute
  - PI: Kevin Weinfurt, Ph.D.
- Evanston Northwestern Healthcare
  - PI: David Cella, Ph.D.

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PROMIS Cancer Supplement Aims

- Increase cancer representation to 2,500 patients with diverse ethnic/racial distributions stratified across the cancer continuum
- Add cancer-relevant domains (illness impact, sexual function, sleep/wake function, and perceived cognitive function)
- Use cancer samples to identify and address challenges related to PROMIS implementation in multi-center oncology clinical trials
- Enhance clinical meaningfulness of PROMIS reporting system for practicing oncologists
- Develop short form instruments for use in NCI-sponsored clinical trials

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Clinical Trials Working Group (CTWG)

- In 2005, The CTWG, chaired by Dr. James Doroshow, released its final goals and recommendations to the National Cancer Advisory Board.
- Priority: Establish a funding mechanism and prioritization process to ensure that the most important correlative science and quality of life studies can be initiated in a timely manner in association with clinical trials.
- The PROACT serves as a platform for informing this implementation process.

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Issues PROACT Will Address

- The collection of HRQOL data in any research setting requires a commitment of resources.
  - Need to define standards and quality in this field to ensure that increased data collection efforts are cost efficient and favorable relative to benefits
  - Goal is improved decision making
- Benefits and challenges of HRQOL data collection may vary by type of clinical trial
  - Most extensive application of HRQOL measurement has been in cancer treatment trials
  - Accelerating application of HRQOL in symptom management trials

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Issues PROACT Will Address

- Recurring challenges and needs:
  - Minimize the resource and administrative costs of HRQOL data collection.
  - Maximize the information value and relevance of the data collected
  - Increase recruitment of diverse racial and ethnic groups in trials
    - Perspectives on HRQOL are diverse
  - How do clinical advances in cancer treatment, prevention, and screening impact the nature and importance of HRQOL assessment in trials?
  - Promote ongoing quality improvement of HRQOL data
    - Reducing missing data is a recurrent challenge
    - Evolving population demographics dictates efforts to improve cultural relevance of surveys

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These challenges framed this conference and today's discussions will help to frame the future -- Help NCI anticipate the future