Enhancing Patient-Reported Outcomes in Cancer Trials:

Taking Stock, Going Forward

Overarching issues for the Panel – and for conference participants:

- Identifying when, where, and how PRO assessment brings significant value to a trial
- Developing guidance for the cost-effective conduct of PRO assessment in trials, to maximize useful information while limiting respondent and administrative burden
- Specifying a research agenda in cancer outcomes measurement and evaluation that is responsive to a range of decision maker needs

Topic Areas

1. The Decision to Collect PRO Data in a Trial
2. Planning the Data Collection and Analysis
3. Field Operations – What Worked? What Did Not? Why?
4. PRO Data Analysis, Interpretation, and Reporting
5. Enhancing PRO Decision Relevance – to the patient, survivor, provider, payer, regulator (may link closely with 1. and 4.)
6. Strengthening support in the larger cancer community (with additional focus on advocacy organizations)
7. NCI's Role in PRO Development and Effective Application in Cancer Trials

When, and how, should PRO measures be included in cancer trials?

- How to determine which specific trials are most suitable for PRO application? Where will PRO use have the biggest “bang”? Describe a prototype situation (the “ideal storm”) where PRO application is most important?
    1. For Phase III trials?
    2. For Phase II / I trials?
    3. For Symptom Management trials?
    4. Pediatric cancer trials?
- How do/should stakeholder interests play into choice of PRO use in trial? Informing choices by patients, survivors, providers, payers.

When, and how, should PRO measures be included in cancer trials?

- Should a given Cooperative Group have a systematic/standardized policy for PRO use? Should PROs be used in every trial? Or should PRO use be investigator-initiated?

- The session on the NCIC and EORTC points out how a large unified cooperative group is able to standardize and obtain buy-in for conducting PRO/HRQOL studies. What are the pros and cons of trying to better integrate PRO/HRQOL studies into clinical trials across 9 separate U.S. cooperative groups?

Effective Planning and Application of PRO Measures in Cancer Trials

- How can the decisions *about PRO use* in a trial be better integrated into Cooperative Group's planning of the entire trial? How to be "at the table" from the beginning?

- What institutional incentives or sanctions (carrots or sticks) would be effective in ensuring PRO data collection & analysis in cancer trials proceeds with the same resolve as for biomedical endpoints? (Attacking the missing data problem!)

Effective Planning and Application of PRO Measures in Cancer Trials

- Are there ways that Cooperative Groups could benefit from a greater sharing of resources:
  * Questionnaires, data collection techniques, training manuals and videos?
  * "Best practices" and "worst practices" in collecting and analyzing PRO data?

- What can U.S. Coop Groups learn from
  * Canada?
  * Europe?
  * Industry?

PRO Analysis, Interpretation, and Reporting

- Are the standard statistical models and techniques that are applied to biomedical endpoints sufficient for PRO analysis?
- Potential role of item-banking and computer-adaptive testing (CAT) in cancer trials? Can we make good on the PROMIS?
- Are we comfortable and confident, yet, in defining a "clinically meaningful difference" in a PRO measure?
- To what extent should FDA Guidance for industry-conducted trials be embraced by Coop Groups?
- How to ensure that PRO findings are reported & published in adequate detail in conjunction with "main" study findings?

Strengthening PRO Support in the Larger Cancer Community

In particular, how can patient and survivor advocacy organizations:

* Encourage decision makers to include the patient's voice in determining the "most effective" cancer intervention?
* Increase recruitment of diverse racial & ethnic groups in cancer trials?
* Build support for enhanced application of PRO measures in cancer trials?
* Building support for initiatives to train the "next generation" of PRO-sensitive (PRO-active?) cancer trialists and outcome researchers?
* Transmitting trial findings – from the patient's perspective – to the cancer community.

NCI's Role in PRO Development and Effective Application in Cancer Trials

- NCI's Clinical Trials Working Group (CTWG) has urged greater attention to quality-of-life studies in NCI-supported trials.
- In response, NCI is forming a "Symptom Management and HRQOL Steering Committee."
- What questions & issues should the working group address? What should be the agenda for its very first meeting?

In sum……
What would be ideal?

- If we could “snap our fingers” and make 3 things happen that would improve the technical quality and decision relevance of PRO measures in cancer trials, they would be:

  1.

  2.

  3.

…..and with all of this in mind, let us *PROceed* to adjourn.