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The Effect of Coverage and Payment on Clinical Research Study Participation and Retention

National Public Forum
September 20, 2007

Background

- Evidence-based practice depends on high-quality clinical trials to generate good evidence.
- Good evidence is generalizable: Clinical trial populations must be representative of real patient populations.
- Generalizability is compromised if people do not have equal access to clinical trials.

Background

- Many factors influence a patient's decision to participate, and to complete, a clinical trial.
- Third party payment policy may influence a patient's ability, and/or decision, to participate in a clinical trial and continue to the trial's end.
- Today, we will discuss this possibility.

Why do patients participate in clinical trials?

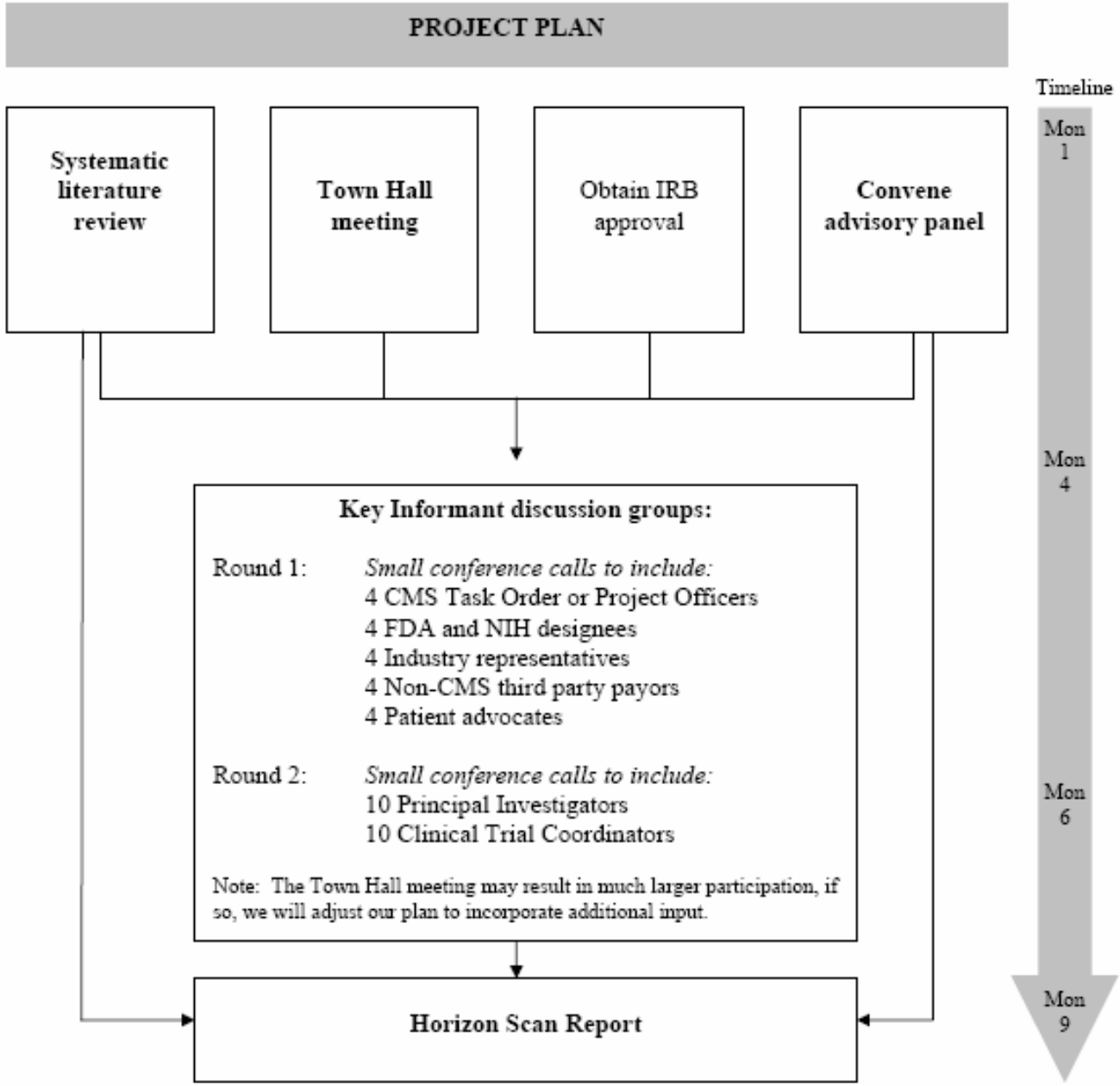
- To gain access to a new treatment that is not available outside of the study;
- To potentially improve their treatment outcomes;
- To contribute to the advancement of medical science and the improvement of available therapies.

What are “down-sides” of participating in clinical trials?

- Expense
  - Sometimes the study provides an opportunity to have the new treatment paid for, but other times it does not.
  - New treatments can be costly.
- Lack of control
  - In “randomized” studies, participants have an equal chance of receiving the (a) new treatment, or (b) current standard of care.
  - Patients cannot specify which treatment they receive.

Possible impact of payment policy

- Health funders (e.g., Medicare, private insurance) sometimes decide to cover treatment for patients who are *not* involved in the clinical trial – but not to reimburse patients who are on the trial – after a study has begun.
- This change in payment policy may affect patients' willingness to enroll, or continue, in the trial.
- Insufficient or non-representative participation in clinical trials may impede collection of data about the benefit/harm of new treatments, and about how they compare to older treatments.


PROJECT PLAN
Timeline
Mon 1
Mon 4
Mon 6
Mon 9
Systematic literature review
Town Hall meeting
Obtain IRB approval
Convene advisory panel
Key Informant discussion groups:
Round 1:
Small conference calls to include:
4 CMS Task Order or Project Officers
4 FDA and NIH designees
4 Industry representatives
4 Non-CMS third party payors
4 Patient advocates
Round 2:
Small conference calls to include:
10 Principal Investigators
10 Clinical Trial Coordinators
Note: The Town Hall meeting may result in much larger participation, if so, we will adjust our plan to incorporate additional input.
Horizon Scan Report

Key Questions

- How do payment policies set by CMS and other payors affect enrollment into clinical trials?
- How do these payment policies affect randomization and blinding within clinical trials?
- What is the aggregate impact of these effects?
- Does the timing of third-party payment in the clinical trial process impact the development of good evidence?
- Do differing payment structures within clinical trials affect the resulting evidence?

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Today's Goal

- To gather information on how payment policies affect real people who might actually participate in clinical trials.
  - Data
  - On-the-ground experiences
  - Diverse viewpoints

NOTE: This is a public forum. All members of the public: lay, industry, government, research, health and non-health related, have equal opportunity to participate.

Questions for the Public

- Would you be willing to participate in a clinical trial if it were the only way to get a new, and possibly more effective, treatment?
- Would you be willing to participate in a clinical trial if you would have to pay out-of-pocket for the drug/procedure while on-trial? If you could receive reimbursement for it off-trial, how would that influence your decision? Why or why not?
- If you decided to enroll in a clinical trial comparing two treatments, and one of the two treatments would cost more than the other, would you be willing to be randomly assigned to *either* treatment?

Questions for the Public (cont.)

- If you *might* be assigned to a study group where you do *not* receive the new drug/procedure (but instead receive "standard care"), would you still be willing to participate in a clinical trial? Would your decision be different if you knew that you *might* get the new treatment for free by being in the trial?
- If you have been involved in the conduct of a clinical trial (e.g., as an investigator, coordinator, manager, or administrator), have you felt that patients' decisions to enroll or continue participation in the trial were influenced by payment policy considerations?
- If you rely on the output from clinical trials to make decisions regarding healthcare (e.g., as a part of evidence-based care), have you felt that the output from clinical trials was influenced by payment policy considerations?

Today's Plan

- Speakers 1-3
  - Dr. Placido Grino: Why do people participate?
  - Dr. Cary Gross: What does the evidence say thus far?
  - Dr. Lori Williams: What is it like to conduct these trials?
- Q/A and public comment

- Speakers 4-5
  - Drs. Lance Dworkin and Daniel Martin: Examples of trials facing these problems
- QA and public comment

- Speakers 6-8
  - Dr. Neil Bressler: TBA
  - Dr. Walter Koroshetz: TBA
  - Dr. Armin Weinberg: Impact of these policies of minority enrollment
- QA and public comment

Process notes

- Each speaker for 7-10 min - Q&A opportunities
- Discussions limited to 7 min -in person/phone
- Will not specifically address the Medicare Clinical Trial Policy under reconsideration.
- Not all issues posed for discussion will become part of our report to CMS.
- If tangential topics are raised, we will record them and convey them to CMS.
- Outcomes of the meeting will be publicly available.

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Responses to Federal Register announcement of Public Forum

- 7 letters received
- Predominantly in area of cancer
- Variety of concerns expressed, with several common themes

Representatives of the following organizations responded:

- Cancer Research Services, US Oncology
- Association of Community Cancer Centers
- American Society of Clinical Oncology (ASCO)
- Moses Cone Regional Cancer Center
- Medical Device Manufacturers Association
- Community Advisory Group, Research Section, San Francisco Department of Public Health
- UPMC (University of Pittsburgh Medical Center) Cancer Centers and the University of Pittsburgh Cancer Institute

Main topics

- Uniform coverage and payment policies are needed.
- Payment policies significantly affect clinical trials' ability to enroll patients.
- Financial barriers are especially acute among Medicare Advantage patients, are unevenly distributed, and result in unequal access to trials and new treatments.
- Clarifications (e.g. of review process) and practical strategies for CMS' implementation of new policies are critical.
- Significant concerns surround the impact on clinical trial enrollment of CMS's proposed (July 2007) change to the 2000 NCD.
- Burden on hospitals and clinical trials sites needs to be examined in light of proposed payment policy changes.
- Timeliness of payment is important to ensure unimpeded progress of research.