Patient and Consumer Advisory Committee

Meeting Synthesis and Committee Recommendations

July 15, 2009

Participants

Maureen Corry	**Executive Director, Childbirth Connection**
Jessie Gruman (Chair)	**President, Center for the Advancement of Health**
Eugene J. Kazmierczak	**FDA Patient Consultant/ Cancer Patient Advocate**
Lawrence Sadwin	**President, Friends of the World Heart Federation Foundation**
Jennifer Sweeney	**Director, Americans for Quality Health Care**
Patience H. White	**Chief Public Health Officer, Arthritis Foundation**

Summary of Key Recommendations

Priority Setting

1. Vet top 10 technologies with relevant patient advocacy groups
2. Create a list of criteria for priority-setting that reflect the values of the U.S. public
3. Provide patient-centered summarized background materials to patient participants
4. Include two public representatives from relevant advocacy organizations on each committee

Effectiveness Guidance Documents

1. Semi-structured interviews should be conducted initially, supported by unrestricted funding
2. Public representatives should review and give feedback on think tank proceedings
3. Create a patient version of the recommendations for representatives to review
4. CMTP should not concern itself with distributing the final results to the public

General Updates on CMTP: Sean Tunis presented an overview of CMTP's work within the past year, and addressed why the patient perspective is important for our work in Comparative Effectiveness Research (CER). CMTP's mission is to facilitate the creation of better evidence through the engagement of a diverse range of stakeholders, and rather than doing systematic reviews, we work to generate new information using prospective studies. This aligns well with the evolving aims of the national CER agenda, which is to generate needed evidence for patients, physicians and payers that will help them make more informed decisions regarding the use of medical technologies.

For many of its projects, CMTP convenes experts and stakeholders from a wide range of backgrounds. However, CMTP needs to further develop ideas on how to more effectively involve patients and consumers and capture their perspectives in a meaningful, pragmatic manner. To that end, the goals of this meeting are to receive concrete feedback about two fundamental pieces of CMTP's work: (1) Priority-setting and topic selection and (2) the development of Effectiveness Guidance Documents (EGDs).

Priority-Setting/Topic Selection: CMTP staff presented the steps of the formalized priority-setting process and our pilot effort in the disease area of cardiology, with a particular emphasis on steps that involve the input of patients, consumers, and other public representatives.

1. **Soliciting Topic Nominations from Public Representatives**

   PCAC came to a consensus that CMTP should not continue using the online topic nomination form as the sole method of soliciting topic suggestions from the public. For the pilot priority-setting effort, CMTP sent a general email to our broad list of contacts asking them to visit the online form and submit a topic and/or distribute the link to their own contacts. PCAC members believe it is difficult and uncomfortable for someone with little or no knowledge of CMTP's work to understand the purpose of the topic nomination process and how the information will be used. Participants also noted that few patients or consumers will have the quantity and quality of knowledge needed to be informed about emerging technologies. They may also be anxious submitting information on the internet if they are not familiar with the organization.

Recommendation: Vetting Top 10 Technologies with Relevant Patient Advocacy Groups.
PCAC members suggested seeking input on technology topics from multiple disease-relevant patient or consumer advocacy groups, instead of focusing on soliciting online topic submissions from individual public representatives. This step would be done once CMTP generated a narrowed list of technologies (a list of ten, typically), and we would pose questions to these organization such as "What is missing from these technologies?" and "What should be considered as a set of priorities?"

2. Criteria Used for Topic Selection

After reviewing the list of criteria that CMTP uses to evaluate potential technology topics, committee members were asked to consider how effective the criteria are at encapsulating the public's values and perspectives on health technology research. Jessie Gruman, backed by other PCAC members, stated that patients do not understand CER and CMTP's mission enough to be able to enumerate criteria that would serve this purpose.

Justine Seidenfeld presented findings from a Canadian study on patient-developed criteria for prioritizing topics for health care research. After noting the similarities between this list and the CMTP criteria, she pointed out that the only difference between the respective criteria was that CMTP did not consider the presence or need for data on adverse events. PCAC members also noted that, while 'cost' was listed as a criterion for Canadians, it is less relevant and appropriate in the context of the U.S. health care system. Participants generally agreed that CMTP's criteria adequately represent public interests, but it would be worthwhile to conduct a more systematic investigation of this issue.

***Recommendation**: Criteria for Priority-Setting That Reflect the Values of the U.S. Public*.
It was suggested that CMTP should consider conducting a project to develop a list of priority-setting criteria that reflect the values of the U.S. public. The PCAC and CMTP staff agreed that this would be of enormous value to other healthcare research organizations, such as the federal government and the IOM. Furthermore, a number of related healthcare advocacy organizations would likely be interested in funding such a project.

3. Using "Patient-Centered" Background Materials

PCAC feels that CMTP should consider utilizing the services of organizations that can provide easily consumed summaries for patients/consumers. This might allow patients to become involved in more of the complex issues related to the technology without having to consume and understanding all of the complexity. In any document that patients and consumers receive, straight-forward, patient-centered language should be used. A good example of this in practice occurred in the cardiac priority-setting process in the spring of 2009, in which Larry Sadwin received less-clinical, summarized versions of the 10 technology briefs.

***Recommendation**: Provide Patient-Centered Summarized Documents to Patient Participants*
This may occur through the use of an outside consultant, or in-house as occurred in the 2009 cardiac technology priority-setting process.

4. Public Participation in Expert Workgroup Meetings

PCAC reviewed composition and responsibilities of CMTP's topic-selection workgroups, using the pilot effort in cardiology (convened in the spring of 2009) as an example. Larry Sadwin served as the public representative for that meeting and gave positive feedback on his overall experience. He said he felt that his opinion was taken seriously by the other members of the group, and noted that asking the right questions of other members was his way of bringing patient considerations to the table. CMTP staff regarded his inputs as very valuable and influential on the priority-setting proceedings.

Several PCAC members acknowledged that the average consumer is not prepared to prioritize research for CER. Mr. Sadwin stated that, given his non-clinical background, some of the materials were difficult to digest and he did not have as much to offer as he would have liked. Jennifer Sweeney suggested that public representatives should be nominated by a disease-relevant advocacy organization, which was supported by

Mr. Sadwin and others. She also suggested, in keeping with Mr. Sadwin's experience, that public representatives work with their respective advocacy organizations to formulate important questions and thoughts for the topic-selection workgroup prior to the in-person meeting, based on given background materials. Dr. Patience White suggested that at least one representative should be a patient with the disease of interest as they bring a more informed viewpoint and can speak from their own treatment experiences.

There was consensus that having just one public representative is insufficient. Being significantly outnumbered by clinicians and others in a workgroup, one representative might feel reluctant to speak freely and openly. Moreover, it would be advantageous to have multiple patient representatives because their experiences differ, and it would result in richer, more comprehensive representation of patient concerns.

***Recommendation:* Two Public Representatives from Relevant Advocacy Organizations.**
CMTP should include two public representatives in each topic-selection workgroup in order to represent a broad patient perspective. PCAC also suggests that representatives should be current or former patients with the disease, and/or should represent a large disease-focused advocacy organization, preferably as part of the organization's leadership structure. The representatives should be encouraged to work with others in their organizations to formulate questions before the in-person meeting, based on the provided background materials.

Effectiveness Guidance Documents: CMTP staff outlined the purpose and scope of Effectiveness Guidance Documents (EGDs), and PCAC members were given the opportunity to clarify their understanding of the product and its development process. Staff then reviewed the steps that involve public participation in order to get input from the PCAC on how to improve the process.

1. **Soliciting Patients' Information Needs to Craft the Initial Recommendations.**

   CMTP introduced a proposed modification of the EGD process to include a step where we conduct interviews with group of decision-makers to elucidate their evidence needs upfront, and these could be used along with other background information to craft the initial draft of recommendations. The purpose of this step would be to engage decision-makers from the very beginning of the EGD development process and ensure that their needs and values are meaningfully considered. As such, CMTP sought input from the PCAC for best practices to solicit evidence needs from members of the public.

   PCAC advised that CMTP should use semi-structured interviews with approximately 5-10 public representatives, rather than conduct focus groups to elucidate evidence needs. Patients are likely to feel more comfortable discussing their experiences in a one-on-one interview. Questions should be tailored to the specific technology, and address quality-of-life issues and other outcomes related to the use of the technology. PCAC will help CMTP develop a list of questions that would be suitable for these types of interviews. Moreover, PCAC urges CMTP to use unrestricted funds to support this kind of work, in order to legitimately maintain a neutral, unbiased point of view.

***Recommendation*: Semi-Structured Interviews Should be Conducted, Supported by Unrestricted Funding.**
Semi-structured interviews should be conducted early in the EGD process and will result in better feedback from patients than focus groups. The questions used should be highly focused on gauging the patient's quality-of-life while using the technology, and a summary of these interviews should be submitted back to participants for their feedback and final approval. It should be made clear to patients that the work resulting from these interviews is funded from unrestricted donations.

2. Public Participation in Expert Advisory Stakeholder Workgroup (ESAW) Think Tanks

PCAC members feel that asking patients to participate in CMTP's ESAW think tanks (as currently structured) is inappropriate, given the complex clinical information that is typically discussed at these meetings. Jessie Gruman and Larry Sadwin noted how marginalized they felt at a previous CMTP think tank on cardiac imaging technologies. Ms. Jennifer Sweeney offered to distribute a guideline on holding multi-stakeholder meetings with meaningful public representation that had recently been produced by the Americans for Quality Health Care program. They suggest holding the think tank, but also asking the public representatives and advocacy organizations associated with the project to review the meeting summary and other products stemming from the meeting to highlight areas of public concern and particular interest. PCAC members were divided on the issue of whether it is necessary to also find a public representative to participate at the think tank meeting.

It was also suggested that CMTP can contract with editors from the Foundation for Medical Decision Making to participate in think tanks as clinically informed experts who will meaningfully provide the public perspective. If so, these FMDM representatives could also review transcripts of the semi-structured interviews with patient representatives (described above) to ensure that CMTP pulls out all of the key guidance that can be used to craft the initial recommendations.

Recommendation: Public Representatives Review and Give Feedback on Think Tank Proceedings.
CMTP should find appropriate public representatives, perhaps from the FMDM, to review think tank notes and give feedback that represents the public perspective- the issue of whether these representatives should additionally participate at the in-person think tank, and in what capacity, remained unresolved.

3. Feedback on Drafts of the Effectiveness Guidance Documents

PCAC members agreed that public representatives need not be given the full draft of the EGD for review, as the highly technical nature of the document would be overwhelming, and most would not be able to give feedback on many aspects of the recommendations. They suggested that CMTP create a simplified version of the recommendations, particularly focusing on those areas of trial design that would be important to patients (i.e. inclusion and exclusion criteria, primary and secondary outcomes, etc.), and give those to the public representatives who have already been involved with the project –the semi-structured interviewees and the think tank reviewers- to review. They recommended that we do not use the online comments survey to get larger patient/consumer feedback, at least in the format that we currently have, as it is too overwhelming. However, the types of patient-specific questions that are listed in the online comments survey would be useful to submit to those who we solicit for feedback on the drafts of the EGD.

Recommendation: Create a patient-oriented version of the recommendations for representatives to review.
CMTP should create a simplified version of the recommendations for trial design, focusing on the parts that are of interest to the public, and give it to public representative who have been involved with previous steps in the project for feedback and review.

4. Dissemination of the Final Product and Results

PCAC members felt that it was not necessary for CMTP to try and distribute the final guidance to public groups, as it would be a greater burden on the organization. They also recommended against CMTP providing guidance to researchers for how to disseminate their (eventual) clinical results to the public. There are a number of advocacy organizations that interpret evidence for the public that would do this on their own, and it would not be the responsibility of the clinical researchers or product developers.

Recommendation: CMTP should not concern itself with distributing the final results to the public.
This can be done better by existing public advocacy organizations, and they ought to be aware of any trials done according to the study design recommendations made in the EGD.

Conclusions and Expanding CMTP's Network of Patient/Consumer Advocacy Organizations: CMTP concluded the meeting by explaining that since a lot of our efforts and projects are centered around specific disease conditions, we need to create better relationships with patient/consumer advocacy organizations in these areas to go to for public representatives and feedback on various project.

PCAC members emphasized the importance of outlining our mission, objective, and purpose for patient and consumer groups to help them understand what we do, how we can serve the public community, and why they should want to work with us. This should also include where various CMTP products fit into the entire clinical research enterprise, and the public's role in CMTP products should be explicitly clear, not assumed.

Next Steps

1. **Review ways to incorporate PCAC feedback into the CMTP's priority-setting and EGD processes**
2. **Create a "scope of work" proposal for a survey research project to identify patient-oriented criteria for priority-setting, and submit for PCAC review**
3. **Create materials for CMTP to use when starting work with patient and consumer oriented groups, including an elevator speech, and submit for PCAC review**

CMTP-Affiliated Participants: Sean Tunis, Leslye Fitterman, Penny Mohr, Russ Montgomery, Justine Seidenfeld, Amie Shei, Merianne Tiglao