BIO
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

September 17, 2007

BY ELECTRONIC SUBMISSION

Michelle Atkinson
Executive Secretary for MedCAC
Centers for Medicare & Medicaid Services
OCSQ-Coverage and Analysis Group
C1-09-06
7500 Security Blvd.
Baltimore, MD 21244

Re: Medicare Program; Meeting of the Medicare Evidence Development and Coverage Advisory Committee (MedCAC)—October 22, 2007

Dear Ms. Atkinson:

The Biotechnology Industry Organization (BIO) appreciates this opportunity to comment on the Centers for Medicare and Medicaid Services' (CMS) public meeting of the Medicare Evidence Development & Coverage Advisory Committee (MedCAC) entitled, "Evidentiary Priorities for the Medicare Program." CMS states that the purpose of this meeting is "to assist CMS in developing priorities for evidence development for issues of major importance to the Medicare program and the Medicare population" and that the end result will be a list of priority research topics with the most potential impact on the Medicare program and beneficiaries.[1]

BIO is the largest trade organization to serve and represent the biotechnology industry in the United States and around the world. BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers, and related organizations. BIO member companies are strongly committed to increasing the body of evidence available regarding diseases and their treatments. Our members invest millions of dollars each year on clinical studies, both before and after Food

[1] 72 Fed. Reg. 48652, 48653 (Aug. 24, 2007).

1201 Maryland Avenue, SW • Suite 900 • Washington, DC 20024-2149 • 202.962.9200 • Fax 202.488.6301 • www.bio.org

and Drug Administration (FDA) approval of their products, to produce high-quality clinical evidence to support medical decision-making. We also support the dissemination of this evidence to advance clinical knowledge and enhance and improve the clinical decision-making process.

Our industry's commitment to developing evidence extends far beyond studies of a particular therapy. We support a rigorous evidence development processes that encompasses all aspects of a disease from examining how it affects the body to studying the cost and benefits of therapies. Our research initiatives advance the understanding of disease pathology and therapeutic mechanisms of action, clinical effectiveness in naturalistic settings, health related quality of life, and health economic impacts of therapies, in addition to clinical safety and efficacy. The development and evaluation of therapies are part of this broader process and must be considered in context.

In holding this MedCAC meeting, CMS raises important questions regarding the diseases, treatments, and health care delivery processes that impact Medicare beneficiaries' health outcomes. However, CMS' intentions in developing these research priorities, and how this MedCAC relates to the Medicare coverage process, are unclear. If CMS endeavors to expand its role into supporting and advancing clinical research under the Medicare program, it must do so through transparent processes that allow for meaningful and continued input from all stakeholders. In general, BIO urges CMS to ensure that any effort to set clinical research priorities does not have unintended consequences for the development of advanced treatment options for the Medicare population. By establishing research priorities without clearly making its intentions known, CMS risks creating uncertainty about coverage and reimbursement that could discourage further investment in research to improve the quality of care provided to all patients.

Furthermore, BIO is concerned that CMS appears to be taking on a task that is outside its authority and unnecessarily duplicates the work of other agencies. We believe CMS should not attempt to set a research agenda for the scientific community, especially when other agencies clearly have responsibility for this task and already have performed it. If CMS chooses to pursue this goal, however, it must not consider cost in any coverage decisions that result from this process. The questions CMS has posed to the MedCAC indicate that the agency is including cost as a factor in setting

priorities. To do so would be an inappropriate deviation from the agency's longstanding policy, and we request greater clarity from CMS on this issue.

I. Research Priorities Should Be Established Through Transparent Processes that Allow for Meaningful Stakeholder Input

BIO agrees that identifying gaps in medical knowledge and fostering research into unmet clinical needs are worthy and important goals. However, we are concerned that the notice of this MedCAC meeting does not clearly describe what CMS will do with the priorities set during this meeting, how this exercise will affect current or future Medicare coverage decisions, and CMS' relation to the missions and responsibilities of other agencies, such as the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH). The clinical research structure is far more complex than CMS may imagine, and it cannot be expanded successfully without the participation of all of its stakeholders, including patients, providers, researchers, manufacturers, and other government agencies. We appreciate CMS' efforts to involve the NIH, Centers for Disease Control and Prevention (CDC), and AHRQ in the discussions at this meeting. These agencies' participation does not help to alleviate confusion about CMS' intentions, however.

Because continued investment in the development of new therapies requires a stable and predictable reimbursement environment, this lack of clarity and transparency could impede access to future improvements in care. CMS' efforts to create a new "framework for the scientific community advancement of research projects that concern Medicare beneficiaries"[2] could disrupt investment and discourage use of new technologies by creating uncertainty about coverage and reimbursement and interfering with private market research priorities, slowing the development of new therapies. BIO urges CMS to exercise caution in pursuing any far-reaching agenda-setting activities and to ensure that any decisions it makes will not discourage research and development of new treatment options.

[2] 72 Fed. Reg. at 48653.

II. CMS Should Not Duplicate the Roles and Responsibilities of Other Agencies in Setting a Research Agenda

BIO believes that setting a research agenda through the MedCAC is not a proper exercise of CMS' authority. Under its authority as a payer, CMS may examine whether a specific item or service meets the criteria for coverage outlined in the Social Security Act (SSA). It is unclear how setting a research agenda to "provide a framework for the scientific community interested in developing evidence that will impact the health of Medicare beneficiaries" falls within the agency's purview.

BIO is concerned that, by engaging in this activity, CMS would inappropriately be duplicating the roles and responsibilities of other agencies, such as AHRQ and NIH. For example, in CMS' guidance on "Coverage with Evidence Development," the agency introduces the "new concept of conducting research under section 1862(a)(1)(E) of [of the SSA to add to the existing body of medical evidence."[3] Section 1862(a)(1)(E) prohibits payment for any item or service that is not reasonable and necessary to carry out the purposes of Section 1142 of the SSA. Section 1142 specifically instructs AHRQ to conduct and support research regarding the outcomes, effectiveness and appropriateness of health care services and procedures;[4] ***establish priorities for the diseases, disorders, and other health conditions for which the research will be conducted***;[5] and develop treatment-specific or condition-specific practice guidelines to improve the quality of care provided[6] (emphasis added).

In addition, Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) authorized AHRQ to establish the Effective Health Care program to evaluate the "outcomes, comparative clinical effectiveness, and appropriateness of health care items and services" provided to Medicare beneficiaries. In carrying out this provision, AHRQ undertook its own research priority setting process, developing a list of priority areas for research under this program

---

[3] Guidance for the Public, Industry, and CMS Staff: National Coverage Determinations with Data Collection as a Condition of Coverage: Coverage with Evidence Development, July 12, 2006, available at: https://www.cms.hhs.gov/mcd/ncpc_view_document.asp?id=8.
[4] SSA § 1142(a)(1)(A)
[5] SSA § 1142(b)(1)
[6] SSA § 1142(a)(3)(A)

specifically targeted to Medicare beneficiaries.[7] We see no reason for CMS to dedicate its limited resources to duplicating the work already performed by AHRQ. We urge CMS not to assume responsibility for a task that Congress clearly has assigned to another agency.

III. CMS' Consideration of Cost in the Coverage Decision-Making Process is Inappropriate and Contrary to Longstanding Agency Policy

BIO also notes that one of the MedCAC panel voting questions asks about the diseases and treatments that are the "costliest to the Medicare Program." The consideration of cost would be contrary to CMS' own statements about the factors considered in making Medicare coverage determinations. According to CMS' own description of the coverage process, when the agency considers whether to cover an item, it looks at the item's clinical characteristics, not its cost.[8] "The cost of an item or service is not relevant in the determination of whether [a] technology . . . should be covered for the Medicare program."[9] Additionally, although CMS expressed intent to use cost as a factor in making coverage determinations in the past,[10] it has abandoned its efforts to establish a rule to implement such a policy. It would be inappropriate for CMS to begin to consider cost in coverage decisions now, particularly without a more meaningful and open dialogue with stakeholders.

IV. The MedCAC Is Not Designed to Answer the Questions Posed to It for This Meeting

We also are concerned that MedCAC is not the appropriate venue to address such broad and far-reaching issues. The MedCAC's charter says that the Committee's purpose is to provide "guidance and advice to CMS on specific clinical topics under review for Medicare coverage."[11] The

---

[7] AHRQ, Effective Health Care Program Priority Conditions, available at: http://effectivehealthcare.ahrq.gov/aboutUs/index.cfm#Conditions.
[8] Guidance for the Public, Industry, and CMS Staff: Factors CMS Considers in Opening a National Coverage Determination, April 11, 2006.
[9] Id.
[10] 54 Fed. Reg. 4302 (Jan. 30, 1989); 64 Fed. Reg. 22619, 22620 (April 27, 1999); 65 Fed. Reg. 31124 (May 16, 2000).
[11] Charter, Medicare Evidence Development & Coverage Advisory Committee, Oct. 17, 2006 (emphasis added).

description of the Committee's functions also indicates that the panel is intended to review specific topics, not to comment on the full range of current and potential Medicare services during a single meeting. The Committee's functions include "review[ing] and evaluat[ing] medical literature, review[ing] technology assessments, and examin[ing] data and information on the effectiveness and appropriateness of medical services and items that are covered under Medicare."[12] Here, instead of reviewing data for a specific item, service, or condition, the panel is being asked to comment on the potential lack of data for every treatment or condition covered by Medicare.

In particular, the questions to the panel require it to comment on "the greatest deficits in knowledge" about a group of diseases and treatments that have not yet been identified.[13] Additionally, although the Charter includes "advising CMS as part of coverage evidence development activities" as one of the panel's functions, it also notes that "each Committee meeting will deal with one or more specific clinical topics."[14] Therefore, any advice on evidence development should be address toward specific clinical topics. The extraordinarily broad subject of this meeting – developing priorities for research affecting the entire Medicare program – cannot reasonably be considered to be "a specific clinical topic."

If CMS wishes to use the MedCAC to identify gaps in knowledge, it should do so by presenting specific conditions or treatments to the panel through the National Coverage Determination (NCD) process. CMS should not use the MedCAC to provide guidance on broad topics, such as the subject of this meeting, because doing so is contrary to the Committee's charter. Asking the MedCAC to comment on this kind of topic also is an ineffective use of the panel's expertise and time. CMS would be better served by convening a panel with expertise on a particular disease or treatment to discuss that specific topic than by asking panelists to address the full range of Medicare services.

Finally, CMS also inhibits the public's ability to comment meaningfully when it puts such broad questions before the MedCAC. As discussed above, BIO firmly believes that transparency and meaningful

---

[12] Id.
[13] Questions to the Panel, http://www.cms.hhs.gov/mcd/viewmcac.asp?where=index&mid=41.
[14] Charter.

stakeholder participation are critical to advancing a clinical research agenda that reflects the needs of patients. With only 30 minutes of scheduled testimony from the public and a 15-minute unscheduled open public session for additional discussion, the agenda of the MedCAC meeting does not allow deep discussion on any one topic that could be raised at this meeting.

V. Conclusion

BIO is committed to increasing the availability of accurate, scientific evidence about diseases and their treatments to inform clinical decision-making, while at the same time, allowing Medicare beneficiaries to have timely access to new innovative therapies. We are concerned that this agenda-setting exercise will impede these goals, and we ask CMS not to duplicate the efforts of AHRQ, NIH, and the private sector to increase the evidence available about new and existing therapies. We thank CMS for the opportunity to raise our issues and concerns and hope that CMS will give thoughtful consideration to our comments. If you have any questions regarding our comments, or would like to further discuss the issues raised, please contact me at 202-312-9281. Thank you for your attention to this important matter.

Respectfully submitted,

/s/

John Siracusa
Manager, Medicare Reimbursement
& Economic Policy