BIO Public Statement: Medicare Evidence Development & Coverage Advisory Committee Meeting

Evidentiary Characteristics for Coverage with Evidence Development (CED)

May 16, 2012

Thank you for the opportunity to present comments on behalf of the Biotechnology Industry Organization, whose members are involved in the research and development of novel interventions to prevent, treat, and cure diseases through the most advanced science. BIO understands that CMS leadership is seeking comments to help develop new guidance on CED and we appreciate the opportunity to provide input on this important policy. My comments today focus on some of the themes raised in the voting questions, and I ask the committee to refer to our written comments, which provide detailed responses to each question.

CMS is interested in whether an evidentiary standard can be defined to invoke CED. BIO believes that there is already a well-established evidentiary threshold applied to coverage of most drugs and biologics. Drugs and biologics are subject to a rigorous evidence-based review by the FDA, and in some cases, post-approval marketing studies. In addition, Medicare and its contractors currently use an evidence-based system to determine coverage for off-label uses of drugs and biologics. By using authoritative compendia and medical literature to define "medically accepted indications," the statute and Medicare's guidance protect beneficiaries' timely access to drugs and biologicals while also ensuring that Medicare's coverage policies are truly evidence-based. CED is best used to expand patient access to therapies that would not otherwise be available, and is therefore not necessary or appropriate for FDA-approved products and medically accepted uses of drugs and biologics..

CMS also is interested in understanding the advantages and disadvantages of non-binary coverage paradigms. The non-binary paradigm has a few notable disadvantages, one being that it creates a substantially less predictable coverage and reimbursement environment. BIO continues to stress, as we have in previous comments, the importance of a predictable, transparent, and clearly defined CED policy. CMS recognized the need to provide for a predictable coverage and reimbursement environment when it first developed principles for applying CED,[1] and this need is still relevant today, and should therefore be reflected in new guidelines. Clarity and predictability are particularly critical for many of BIO's emerging company members who depend on private equity investment to fund drug development. If, under a newly designed CED policy, manufacturers are unclear about the rationale for CMS' application of CED, investment in new medical technologies may be severely interrupted, and patient access to improved drugs and biologicals may be delayed. Therefore, CMS must communicate why CED for a given technology is considered necessary, and identify, *a priori*, the outstanding evidentiary questions required to be resolved. The process for collecting evidence must be understandable, and each data collection activity must have a well-defined end point. BIO believes strongly that CED studies should not be an open and undefined data gathering exercise: CED decisions should have clearly defined timeframes that dictate when sufficient evidence has been collected to support review for full coverage. We acknowledge that

[1] 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. The eight principles governing the application of CED as articulated in the guidance document are: a) National Coverage Determinations (NCDs) requiring CED will occur within the NCD processes, which is transparent and open to public comment; b) CED will not be used when other forms of coverage are justified by the available evidence; c) CED will in general expand access to technologies and treatments for Medicare beneficiaries; d) CMS expects to use CED infrequently; e) CED will lead to the production of evidence complementary to existing medical evidence; f) CED will not duplicate or replace the FDA's authority in assuring the safety, efficacy, and security of drugs, biological products, and devices; g) CED will not assume the NIH's role in fostering, managing, or prioritizing clinical trials; h) Any application of CED will be consistent with federal laws, regulations, and patient protections.

while this timeline may be different for various products or services, it should be part of the ongoing dialogue between CMS, the interested stakeholders, and appropriate expert advisors, such as clinical epidemiologists and scientists. In addition, CMS needs to carefully weigh the additional costs and burdens imposed on stakeholders with the types of studies it considers sufficient for CED purposes.

Finally, any application of CED must be developed in a transparent and predictable manner, with opportunity for stakeholder comment, to ensure that CMS reaches an appropriate decision for patients. To achieve this goal, CED should occur only within the auspices of the NCD process, which has predictable procedures and timelines to establish a valid coverage determination.

BIO again appreciates the opportunity to comment on evidentiary characteristics of CED, and we encourage the Agency and the MEDCAC to continue to solicit input from stakeholders in order to develop a CED policy that is transparent and predictable.