November 23, 2011

VIA ELECTRONIC SUBMISSION

Donald Berwick, MD
Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Ave. SW
Washington, DC 20201

Re: Potential NCD Topics

Dear Administrator Berwick:

The Biotechnology Industry Organization (BIO) appreciates this opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS) Medicare Coverage document entitled "Potential NCD Topics."[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 centers, academic institutions, state biotechnology centers, and related organizations in the United States and in more than 30 other nations. BIO members are involved in the research and development of health care, agricultural, industrial and environmental biotechnology products.

BIO represents an industry that is devoted to discovering and ensuring patient access to new and innovative therapies. Medicare coverage of these therapies is vital to ensuring the health and wellness of many Medicare beneficiaries, and a predictable and transparent Medicare coverage process is essential to providing timely access to appropriate treatment options.

In general, BIO supports CMS's efforts to improve the transparency of the national coverage determination (NCD) process by seeking input on potential NCD topics. We are very concerned, however, that CMS's request for NCD topics refers to a new "minimal benefit" standard for determining whether to issue an NCD that not only is contrary to federal law and the agency's own guidance, but also threatens to restrict beneficiary access to much needed medical therapies. Instead, we urge CMS to follow the "reasonable and necessary" standard set forth under federal law and the agency's own guidance interpreting that standard when seeking potential NCD topics. Furthermore, we urge CMS to seek public comment on any new list of NCD topics that it publishes and consider removing items from the list of potential NCD topics.

[1] CMS, Potential NCD Topics (Sept. 28, 2011), *available at* http://www.cms.gov/medicare-coverage-database/details/medicare-coverage-document-details.aspx?MCDId=19&McdName=Potential+NCD+Topics&mcdtypename=Potential+National+Coverage+Determination+&MCDIndexType=2&bc=AAAEAAAAAAAA&.

I. CMS Should Use Existing Standards When Identifying Potential Topics for NCDs

In the request for potential NCD topics, CMS invites input concerning items and services that "may be inappropriately used (i.e., underused, overused, or misused) or provide minimal benefit in hospitals, clinics, emergency departments, doctors' offices, or in other healthcare settings."[2] These criteria for identifying potential NCD topics are different from the criteria established by statute and CMS guidance. In particular, the "minimal benefit" standard is new, undefined, and conflicts with longstanding guidance on interpretation of the statutory "reasonable and necessary" requirement for coverage.

Under the federal Medicare statute, Medicare payment for most items or services is contingent upon the determination that the item or service falls within a benefit category, is not specifically excluded from coverage, and is "reasonable and necessary for the diagnosis and treatment of illness or injury or to improve the functioning of a malformed body member."[3] CMS has the authority to implement NCDs that identify the circumstances under which items and services are considered to be "reasonable and necessary," as well as to develop guidance documents that explain the factors considered in making NCDs.[4]

We could find no instance in CMS's guidance documents or its coverage decisions where "minimal benefit" has been used or defined as a criterion for developing an NCD for an item or service. Although CMS has not defined "reasonable and necessary" in regulation, in numerous NCDs and CMS's own guidance documents on the development of NCDs, CMS has identified "improved health benefit" as a key standard for determining whether an item or service is "reasonable and necessary."[5] In addition, in statements to the medical community, CMS has said that it uses the following definition for "reasonable and necessary:" "adequate evidence to conclude that the item or service improves clinically meaningful health outcomes for the Medicare population."[6] This definition requires only that the item or service improve health outcomes; it does not attempt to establish a minimum level of improvement necessary for coverage.

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[2] Id. (emphasis added).

[3] Social Security Act (SSA) § 1862(a)(1)(A). Coverage of certain other services, such as certain preventive services, is provided under other subparagraphs of § 1862(a)(1) or other specific provisions of the SSA.

[4] SSA § 1862(*l*)(1).

[5] CMS, Factors CMS Considers in Opening a National Coverage Determination (Apr. 11, 2006), available at: http://www.cms.hhs.gov/mcd/ncpc_view_document.asp?id=6; CMS, National Coverage Determinations with Data Collection as a Condition of Coverage: Coverage with Evidence Development (July 12, 2006), available at: https://www.cms.gov/medicare-coverage-database/details/medicare-coverage-document details.aspx?MCDId=8&McdName=National+Coverage+Determinations+with+Data+Collection+as+a+Condition+of+Coverage*3a%24+Coverage+with+Evidence+Development&mcdtypename=Guidance+Documents&MCDIndexType=1&bc=AgAEAAAAAAAA&; see also, e.g Decision Memo for Magnetic Resonance Imaging (MRI) (CAG-00399R3), July 7, 2011; Decision Memo for Autologous Cellular Immunotherapy Treatment of Metastatic Prostate Cancer (CAG-00422N), June 30, 2011; and Decision Memo for Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) for Myelodysplastic Syndrome (CAG-00415N), August 4, 2010.

[6] See, e.g., Barry M. Straube, MD, CER, Personalized Medicine & Coverage, October 20, 2010, at 7, http://conferences.thehillgroup.com/CERandPMconference/presentations/Day%202/Day%202_Straube.p

In addition, CMS's guidance on the factors it considers when deciding whether to open a national coverage analysis (NCA) do not refer to "minimal benefit" as a criterion for coverage. This notice provides a comprehensive outline of the agency's policies with respect to the NCD process developed after a period of notice and comment. In this guidance, CMS set forth the necessary aspects of a request for an NCD, including:

- A rationale for how the evidence selected demonstrates the medical benefits for the target Medicare population;
- Information that examines the magnitude of the medical benefit; and
- Reasoning for how coverage of the item or service will help improve the medical benefit to the target population.

CMS asks the party seeking an NCD to describe the "magnitude of the medical benefit" of the item or service at issue, but CMS does not define the magnitude necessary to support coverage. The recent request for potential NCD topics diverges from the 2003 notice by suggesting that items and services with only "minimal benefit" might not be covered by Medicare.

CMS provided additional guidance on the criteria that could prompt the opening of an NCA in its April 11, 2006 guidance document, "Factors CMS Considers in Opening a National Coverage Determination," issued on April 11, 2006. This guidance identified the following criteria for an existing technology that already is in use:

- Providers, patients or other members of the public have raised significant questions, that are supported by CMS's initial review of available data, about the health benefits of currently covered items or services, specifically regarding the Medicare population;
- Interpretation of new evidence or re-interpretation of previously available evidence indicates that changes may be warranted in current policies;
- Local coverage policies are inconsistent or conflict with each other to the detriment of Medicare beneficiaries. For instance, the noted variation is not related to local differences in the capabilities of health care providers to use the technology effectively which can be resolved over time, but rather is causing significant disparities in the care available to Medicare beneficiaries that are unlikely to be addressed effectively through provider training and education or through the local coverage process;
- Program integrity concerns have arisen under existing local or national policies; that is, there is significant evidence of wide variation in billing practices not related to variation in clinical need, or of potential for fraud under existing policies.[7]

In addition, CMS identified the following criteria for generating an NCD for "a new item or service, an existing item or service that has been substantially modified, or for a proposed new use of a covered product."

- The health technology represents a substantial clinical advance and is likely to result in a significant health benefit if it diffuses more rapidly to all patients for whom it is indicated.

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df; Tamara Syrek Jensen, Medicare Coverage, November 14, 2008, at 11, http://www.npcnow.org/App_Themes/Public/pdf/events/2008_event/syrekjensen.pdf.

[7] CMS, Factors CMS Considers in Opening a National Coverage Determination (Apr. 11, 2006), *available at* http://www.cms.hhs.gov/mcd/ncpc_view_document.asp?id=6.

- More rapid diffusion of the technology is likely to have a significant programmatic impact on Medicare and on other Medicare-related public policies (e.g., reduction in health inequalities).
- Significant uncertainty exists concerning the health benefits, patient selection, or appropriate facility and staffing requirements for the new technology. The presence of significant uncertainty about benefits and risks is of particular concern when rapid diffusion of the item or service is likely when:
  - Use of the new item or service likely conflicts with existing NCDs.
  - Available evidence suggests that local variation is not warranted.[8]

CMS also specified:

> Cost effectiveness is not a factor CMS considers in making NCDs. In other words, the cost of a particular technology is not relevant in the determination of whether the technology improves health outcomes or should be covered for the Medicare population through an NCD.[9]

These criteria recognize that an item or service might be a good subject for an NCD if there are questions about its benefits, but unlike the "minimal benefit" standard mentioned in the request for potential NCD topics, they do not suggest that there is a threshold level of benefit needed for coverage. Similar to the 2003 Federal Register Notice, the guidance document requires that requests for NCDs provide information "that measures the medical benefits of the item or service,"[10] but CMS has not established a particular amount of benefit that is necessary to support coverage. Under the published criteria for consideration of potential NCD topics and the "improved health outcome" standard, it only is necessary to demonstrate that an item or service has a potential clinical benefit to some Medicare beneficiaries in order for CMS to consider issuing an NCD.

If CMS wishes to collect suggestions for topics for NCDs, we ask that it use these criteria, which have been shared with the public and developed after public notice and comment, to identify items and services for which an NCD might be appropriate. By seeking to identify items and services that provide "minimal benefit" as candidates for NCDs, CMS appears to establish a new standard for coverage analysis that is inconsistent with CMS's longstanding interpretation of the Medicare statute's "reasonable and necessary" standard because it suggests that some items and services that improve health outcomes could be denied coverage because they fail to achieve an undefined level of improvement.

Beyond being inconsistent CMS's interpretation of "reasonable and necessary," the "minimal benefit" standard also threatens to hinder beneficiary access to medically necessary items and services. First, it is not clear that the "minimal benefit" standard takes into consideration the needs of specific patient sub-populations. As CMS is aware, the medical needs of Medicare beneficiaries are varied and diverse. An item or service could be beneficial for one patient population but not for another. CMS's longstanding interpretation of the Medicare statute's "reasonable and necessary" standard is able to accommodate this diversity by covering items and services for even the smallest subset of beneficiaries to the extent that such care improves health outcomes for them. The "improved health outcome" criterion also recognizes that the size of an item or service's effect on a patient's health

[8] Id.
[9] Id.
[10] Id.

outcomes may vary depending on the patient's unique condition, and that patients and their physicians are best suited to judge whether the benefits of a particular therapy justify any associated risks. For beneficiaries with few treatment options, a therapy that provides even a small benefit could be worth pursuing. In addition, the "improved health outcome" criterion recognizes that improvements in medical technologies often are incremental, and it allows Medicare to cover items and services that help to improve health outcomes without attempting to establish a minimum threshold for improvement for any patient population that is necessary for coverage. As a result, this criterion permits Medicare to cover items and services that can improve a patient's health outcomes to any degree, and patients and physicians can determine whether the potential benefit for each patient justifies use of that technology. The same cannot be said for the "minimal benefit" standard. This standard suggests that an item or service must show not only that it is beneficial, but also that it exceeds a currently undefined threshold of benefit that is necessary for coverage.

Second, merely publishing a list of technologies that CMS suspects provide "minimal benefit" will have negative implications for access to care. In particular, by posting a list of items and services believed to be of "minimal benefit," CMS may encourage Medicare contractors to cease coverage for these items and services given the negative connotations of this designation, even though CMS has not considered the available evidence or completed the NCD process. Thus, even if CMS ultimately decides to cover an item or service on the list, beneficiaries will not have had access to that benefit in the interim. We believe that this is a serious concern given that that our members have experienced contractors' denials of coverage for their therapies during development of an NCD. BIO urges CMS to provide clear guidance to local carriers instructing them to continue coverage while an NCD is open.

In light of the foregoing, we urge CMS to withdraw the request for comments on items and services that provide "minimal benefit" from the September 28 Coverage Document. Instead, we urge CMS to rely on the Medicare statute's "reasonable and necessary" standard and the clinical benefit standards articulated in the agency's 2003 and 2006 guidance in deciding whether to open an NCD.

II. CMS Should Explain the Basis for Adding to the List of Potential NCD Topics

If CMS develops a list of potential NCD topics based on the September 28, 2011 request for comments, we ask CMS to seek public comment on that list before it is finalized. In addition, BIO requests that CMS identify the origin of each topic recommendation (i.e. CMS headquarters, medical specialty society, patient group etc). In 2008, when CMS published a list of potential NCD topics, it provided a brief description of each topic on the list, but often did not describe the clinical basis for proposing an NCD, cite publications, or discuss the evidence considered by CMS when it placed the item or service on the list. As a result, some of the topics may not have reflected the most recent evidence on the item or service. BIO urges CMS to clarify why a topic may be under consideration for an NCD Stakeholders may be able to provide comments to CMS on a draft list of topics that could respond to questions about the technology or the clinical evidence that could help CMS refine the list or remove items from the list without expending the resources to open an NCA.

In addition, we ask CMS to consider removing an item or service from the list of potential NCD topics, or to revise the description of an item or service on that list, after the initial comment period has ended. As the evidence develops, CMS may find that it does not need to dedicate time and resources to develop an NCD on a topic. Removing topics from the list in these circumstances

helps to clarify CMS's intentions and resolve any confusion among stakeholders about potential changes in coverage for that item or service. BIO asks CMS to develop a clear and realistic timeline for updating the list and to consider removing topics from the potential NCD list without requiring initiation of an NCA.

Lastly, after the list has been created, we ask CMS to ensure that it has the specific internal expertise that is necessary to conduct accurate assessments of each topic. Given the complexity of these issues, it is crucial that CMS enlist the assistance of trained, current, technical experts to ensure that accurate determinations are made in the best interest of patients. As we noted in our comments on the parallel review process, Medicare coverage of most drugs and biologicals is determined appropriately by local contractors. CMS's policies allow contractors to cover both approved and off-label uses of other drugs that are approved by the FDA and are "reasonable and necessary for diagnosis or treatment of an illness or injury."[11] In practice, contractors make coverage determinations for drugs and biologicals in an appropriate and timely manner. Thus, we ask that CMS ensure the same level of expertise in its review of items and services at the national level as it does at the local level.

III. Conclusion

BIO appreciates the opportunity to comment on Potential NCD Topics. We look forward to continuing to work with CMS to address this and other issues in the future. Please feel free to contact me at 202-962-9220 if you have any questions or need any additional information. Thank you for your attention to this very important matter.

Sincerely,

/s/

Laurel L. Todd
Managing Director
Reimbursement and Health Policy

[11] Medicare Benefit Policy Manual, ch. 15, §§ 50.4.1-50.4.3.