December 23, 2004

BY ELECTRONIC DELIVERY

Mark McClellan, M.D. Ph.D., Administrator
Centers for Medicare and Medicaid Services
Hubert H. Humphrey Building
200 Independence Avenue, S.W.
Department of Health and Human Services
Washington, D.C. 20201

Re: Draft Formulary Review Criteria (Medicare Prescription Drug Benefit)

Dear Administrator McClellan:

The Biotechnology Industry Organization ("BIO") appreciates this opportunity to comment on the Centers for Medicare and Medicaid Services ("CMS") Draft Formulary Review Criteria ("Draft Guidelines"), posted on the CMS web site on December 3, 2004, pursuant to the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the "MMA"). 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 1,000 biotechnology companies, academic institutions, state biotechnology centers, and related organizations in the United States. 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 cures. We have long supported extending Medicare coverage to all drug and biological therapies, regardless of how they are administered. Many of the therapies developed by biotechnology companies target conditions that primarily affect seniors. We support the establishment of the Medicare Part D prescription drug benefit and appreciate CMS' efforts to implement this program. We continue to encourage CMS to focus on patient access as it implements the Part D benefit, particularly as CMS considers how best to evaluate plan formularies to ensure that Part D enrollees have meaningful access to these critical therapies.

BIO supports the overall CMS approach to formulary review, which is to rely on best practices in the private sector both in Pharmacy and Therapeutics ("P&T") committee operations and formulary list developments. We support CMS' approach of using private sector benchmarks by which to evaluate the adequacy of Part D plan drug lists. We also appreciate CMS' concern and awareness of the unique characteristics and needs of the Medicare population that differ from the employed, private sector population covered under the proposed benchmarks. The companies that comprise BIO are most keenly aware of the needs of patients with rare conditions and serious illnesses – more prevalent in the Medicare population than in the working age population. It is from this perspective that we have reviewed the Draft Guidelines and provided comment.

I. P&T COMMITTEES

BIO generally supports CMS' proposals in the Draft Guidelines to strengthen and clarify the role of P&T committees. Properly utilized, P&T committees play a critical role in ensuring that a plan's enrollees have adequate access to the full range of medically necessary drugs and biologicals. Our specific comments on the Draft Guidelines proposals regarding P&T committees are as follows:

A. Membership and Meeting Requirements

BIO supports CMS' clarifications on P&T committee membership requirements[1] and the proposals that P&T committees must meet at least quarterly

[1] Draft Guidelines at 5.

and that P&T committee decisions regarding formulary development or revision must be documented in writing.[2] We also support CMS' proposal regarding disclosure of conflicts of interest for P&T committee members.[3] These requirements should be adopted in the final guidelines.

B. P&T Committee Approval of Cost Containment Mechanisms

BIO supports CMS' proposed requirement that P&T committees review for clinical appropriateness the practices and policies for formulary management activities, such as access to non-formulary drugs and biologicals, prior authorization, step therapy, generic substitutions, therapeutic interchange protocols and other drug utilization activities that affect enrollee access to necessary therapies. The role of the P&T committee should be to establish a clinically appropriate formulary. As part of that role, BIO supports CMS' proposal that a plan's P&T committee play a key role in defining these types of policies to ensure that these tools are not used to hinder medically appropriate access to covered Part D drugs.[4]

We are concerned, however, that CMS has not required that a P&T committee actually approve these types of cost containment tools in order for a plan to use them. More specifically, we ask CMS to clarify that the plan's P&T committee actually must *approve* any such restrictions on access as part of the P&T committee's overall approval of the plan's formulary when those restrictions affect the clinical appropriateness of the plan formulary. For example, a P&T committee may determine that, for clinical reasons, it is critical that a particular class of drugs or biologicals include several therapies in the preferred tier. Because this determination is central to ensuring that a plan formulary provides a clinically appropriate range of therapies, the plan should be obligated to abide by this P&T committee recommendation. We would not expect, however, that the P&T committee would play a role in tiering decisions absent specific clinical concerns. Generally, those decisions will be best left to the plan's negotiation and contracting process. Requiring that a plan abide by P&T committee recommendations – including determinations regarding cost containment policies –

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[2] *Id.* at 6.
[3] *Id.* at 5-6.
[4] *Id.* at 4-5.

that are based in clinical considerations will help ensure that the formulary appropriately reflects the clinical needs of Medicare beneficiaries.

C. Changes to Therapeutic Classes

CMS proposes, consistent with the MMA, that the P&T committee must approve inclusion or exclusion of the therapeutic classes in the formulary on an annual basis.[5] BIO is concerned that this standard, as implemented, could allow a P&T committee to eliminate classes on the formulary without CMS approval, and we seek clarification regarding CMS's intent to monitor these changes on an ongoing basis. If a plan is permitted to remove therapeutic classes on an annual basis without approval from or notification to CMS, the result could be that a plan initially adopts a CMS-approved formulary but later makes substantial changes that result in a formulary that fails to provide a comprehensive drug benefit or discriminates against certain groups of Medicare beneficiaries. CMS proposes to approve plan formularies as an initial measure, but it is not clear whether there will be a mechanism for CMS to approve subsequent changes to a formulary, such as the removal of classes. We appreciate CMS' statement that it will "monitor changes to approved formularies on an ongoing basis and initiate discussion when necessary to assure that a formulary remains non-discriminatory."[6] Nonetheless, BIO asks CMS to establish a clear process for monitoring plan formularies and to clarify whether this monitoring will be part of a plan's annual contract renewal process. We also ask CMS to clarify that a P&T committee – and a plan – may not reduce the number of classes or eliminate specific classes without CMS approval or some formal notification to CMS.

D. Formulary Inclusion of New Drugs and Biologicals

BIO supports CMS' proposed requirement that a plan's P&T committee must review each new chemical entity within 90 days of its market release or provide a clinical justification if this timeframe is not met. BIO represents an industry that is devoted to discovering new and innovative treatments and therapies and ensuring patient access to them. Our members continually are developing promising new medicines. It is imperative that these new therapies be available to Medicare beneficiaries in a timely manner so that they may have the advantage of life-saving and life prolonging innovations. We strongly support the

[5] *Id.* at 6.
[6] *Id.* at 10.

requirement that P&T committees consider new therapies within 90 days. We ask CMS to adopt this proposal in the final rule as well as in the final guidelines. We also request that CMS require P&T committees to consider new indications for existing therapies within 90 days of approval of the new indication. In addition, we request that CMS clarify that "new chemical entity" is intended to include biologicals approved under a biologics license application ("BLA").

In establishing the time period for P&T committee consideration of new therapies and new indications, BIO requests that CMS require plans to place new therapies and therapies for which there are new indications on the plan formulary's preferred tier during the period in which P&T committee approval is pending. This reflects the best practices in the industry now and ensures that patients will have appropriate access to life-saving and life-enhancing new therapies. We understand that plans are actively encouraging CMS to extend the proposed 90 day period to six months or longer. Regardless of the timeframe that CMS implements in the final guidelines, it is critical that plan formularies provide open coverage of therapies during this period, consistent with current best practices.

Finally, we ask CMS to clarify when a plan may justify a delay based on clinical reasons. Absent unusual circumstances, such as the release of substantial new clinical information within a few days prior to the 90 day deadline, we believe this exception should not be used. BIO would appreciate CMS providing additional guidance on the appropriate use of this exception. Otherwise, a P&T committee could routinely state at the 90 day deadline that it needs more time for clinical reasons, undermining CMS' efforts to have new therapies available to Medicare beneficiaries in a timely manner.

E. Formulary Exceptions

We are concerned that CMS may be proposing to give P&T committees too much discretion in establishing procedures for enrollees to access non-formulary drugs and biologicals. The Draft Guidelines require P&T committees to establish protocols and procedures for the timely use of and access to both formulary and non-formulary drugs and biologicals.[7] As CMS acknowledges, an enrollee may need a non-formulary drug or biological when the formulary drug or biological would cause adverse effects, would not be as effective,

[7] *Id.* at 7.

or both, based on scientific evidence or medical necessity.[8] We are concerned that the exceptions process set forth in the Proposed Rule does not provide adequate access to medically necessary drugs and biologicals. In our comments to the Proposed Rule, we urged CMS to revise this process to ensure enrollees appropriate access to medically necessary therapies by implementing a meaningful exceptions process and by providing for reasonable cost-sharing for those therapies for which an exceptions request is approved. Although we support the proposal in the Draft Guidelines that P&T committees play a role in establishing appropriate procedures for timely access to non-formulary drugs and biologicals, we believe that the P&T committee process alone is not sufficient to ensure enrollees adequate access when an exceptions request is necessary. BIO requests that CMS establish detailed procedures in the final rule for ensuring such access and not rely only on the P&T committee process. Also, we request that CMS provide further guidance for P&T committees on the committee's appropriate role in establishing such procedures.

In particular, we believe it is critical for CMS to establish clear requirements for ensuring that enrollees have access to an emergency supply of medication during an exceptions request and that the timeframe in which plans must respond to exceptions requests reflect the standard for private plans. For the patients BIO members serve – typically those with chronic and severe illnesses who have continuing therapeutic needs – it is critical that the exceptions process not limit the ability to access critical therapies. In many cases it is not medically feasible for an enrollee to stop using a biological therapy and then later re-start the therapy. Also, switching medications routinely requires laboratory tests and physician visits. An enrollee relying on a therapy that is removed from the plan formulary will need time to make this transition. For these reasons, it is critical that a plan both resolve exceptions requests in a timely manner[9] and that an emergency supply of medication is provided during the exceptions process when necessary to ensure continuity of a therapy or to provide a therapy that is urgently needed. Although a P&T committee can provide additional protections for

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[8] *Id.*

[9] We note that many private plans respond to prior authorizations either immediately or within two days. *See, e.g.,* "Drug Prior Authorization," Blue Shield of California, available at https://www.mylifepath.com/bsc/pharmacy/faqs/pharmacy_faqs_drug_authorization.jhtml; "Group Health Insurance Prior Authorization," AmeriHealth, available at http://www.amerihealth.com/jsps/article.jsp?id=/plan_info/group/supplemental/prescription /sup_prescription_prior_auth.html; "Pharmacy Prior Authorization Request Forms," Cigna, available at http://www.cigna.com/health/consumer.service.pharmacy_priorauth.html.

enrollees, we believe it is critical for CMS to establish specific criteria in the final rule that sets forth the minimum standards for a plan's exceptions process.

II. FORMULARY LIST REVIEW

As stated above, BIO generally supports CMS' approach to using private sector benchmarks in determining the adequacy of Part D plan formularies, in conjunction with special consideration of the unique needs of the Medicare population. We believe that an array of private sector benchmarks, as CMS has proposed, is important to ensuring the success of the Part D benefit. As we mention above, BIO has experience and knowledge of the needs of very sick and extremely vulnerable Medicare patients. The needs of these beneficiaries will require special attention under Part D. It is critical that in evaluating and approving formularies, CMS be particularly cognizant of the needs of enrollees with rare diseases and conditions and ensure that the therapies needed to treat these diseases and conditions, including orphan drugs and biologicals or therapies that treat rare conditions, are readily available to enrollees who need them.

Biological therapies pose special formulary concerns. On the one hand, biological therapies tend to be costly and treat populations that present certain challenges to private plans. On the other hand, these treatments often result in dramatic improvements in patients' lives and save Medicare costly expenditures by avoiding surgeries and inpatient admissions. Many biological therapies provide treatment to patients with rare diseases and conditions. By their very nature, these therapies typically are not interchangeable, and thus it is critical that a full range of biological therapies be available to Medicare beneficiaries. For these reasons, the process by which CMS reviews Part D plan formularies to ensure non-discrimination will be particularly critical to Medicare beneficiaries who rely on biological therapies. With this in mind, we comment on the specific formulary review provisions of the Draft Guidelines below.

A. <u>Review of Formulary Classification Systems</u>

BIO supports CMS' proposal, consistent with the MMA, to review both a formulary's classification system and how that classification system is populated. CMS requests comments on whether there are classification systems *other than* the United States Pharmacopeia ("USP") system that should be exempt from CMS' review of plan formulary classification systems. We appreciate CMS'

recognition that such a review of a plan's formulary classification is only one factor in determining whether a plan formulary is adequate for purposes of ensuring enrollees access to a meaningful prescription drug benefit, and we believe this recognition is consistent with the MMA. We would not object to CMS treating other classification systems – those that meet at least the minimum categories and classes of the USP Model – as exempt from review.

As we have expressed in our comments to the Proposed Rule and to the USP Draft Model Guidelines, we are extremely concerned that the USP Draft Model Guidelines will not provide enrollees with adequate access to many drugs and biologicals, particularly those therapies needed by beneficiaries with certain conditions, such as end stage renal disease ("ESRD"), multiple sclerosis, or uncommon diseases and disorders. We reiterate our concerns with the USP Draft Model Guidelines. For example, the Draft Model Guidelines fail to include an adequate category for phosphate binders, required by ESRD patients, despite the fact that all ESRD patients are covered by Medicare, regardless of age. Moreover, therapies required for multiple sclerosis would not fall into any categories or classes in the Draft Model Guidelines. As therapies for multiple sclerosis currently compose more than 25% of Medicare's replacement drug demonstration, access to these therapies is critical for this population. We remain concerned that enrollees may be able to access these therapies only through the exceptions process or the potentially lengthy appeals process. We ask CMS to consider whether the classifications proposed by Part D plans ensure that enrollees with a wide range of diseases and conditions have access to the therapies they need.

B. Appropriate Benchmarks for Use in Evaluating Formularies

We support the use of comprehensive private plan formularies as examples of formularies that tend to provide coverage for a broad range of drugs and biologicals, including those that are critical for Medicare beneficiaries. For example, comprehensive private plan formularies tend to cover *all* HIV and hepatitis medications and numerous cancer therapies, including antineoplastics and immunosuppressives. Nonetheless, we note that even these more comprehensive formularies do not provide adequate benchmarks for enrollees with certain conditions, such as orphan diseases. We ask CMS to review Part D formularies to ensure that a plan design does not discriminate against those beneficiaries with uncommon conditions.

C. Drug List Review

BIO supports CMS' efforts to benchmark each proposed Part D formulary against existing widely used formularies that provide broad coverage for seniors and persons with disabilities. A review of the drugs and biologicals listed on a Part D formulary will help to ensure that Part D plans provide the kind of comprehensive prescription drug benefit available to seniors through many private plans.

We are concerned, however, about CMS' proposed use of lists of the top 25-50 drugs and biologicals for the Medicare population in terms of cost and utilization.[10] Although we support CMS' interest in ensuring that these drugs and biologicals are available to Medicare beneficiaries through the Part D benefit, we wish to emphasize that formulary approval process that relies only on such a list likely will not provide adequate coverage for beneficiaries with rare diseases and critical conditions. Enrollees need appropriate access to drugs and biologicals that treat common diseases and conditions, and as an absolute minimum standard, Part D plans must provide coverage for the therapies most widely used by the Medicare population. Yet this type of benchmark, used in isolation, will not provide the comprehensive benefit that Congress intended in creating Part D and is not likely to protect the most vulnerable of Medicare beneficiaries – those with rare diseases or conditions requiring multiple therapies.

As noted above, a significant percentage of biological therapies on the market are designed to treat rare diseases and disorders, such as Idiopathic Pulmonary Fibrosis or Gaucher's disease. We are concerned that reliance on lists of the top 25-50 drugs and biologicals could fail to ensure that enrollees with rare diseases or disorders with medically appropriate therapies. Even if such lists include some of these types of therapies, they will not include the range of drugs and biologicals to which enrollees will need access. Patients with uncommon diseases and disorders should have the same access to medically necessary drugs to treat their conditions as do patients with common conditions. We appreciate CMS' recognition that it needs to "assess the availability and tier position for commonly prescribed drugs for uncommon conditions."[11] We request that CMS clarify that this assessment will be a critical component of the formulary approval process.

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[10] *Id.* at 9.
[11] *Id.*

We also note that private plans, although they may provide a useful starting point, are not likely to provide a full list of the drugs and biologicals that may need to be included in a Part D plan. For example, although private payers play an early role in the care of many ESRD patients, Medicare is the chief payer overall. Because private payers have had substantially less practical experience with pharmacotherapy for these patients, private plan formularies may not adequately reflect the prescription needs of these patients.

In using private plans as benchmarks, it is important to be aware that private plans typically provide a prescription drug benefit as part of a comprehensive health benefit. As a result, a private plan may choose to cover certain therapies – such as vaccines – as part of a plan's medical benefit rather than as part of the plan's prescription drug benefit. In fact, of the five formularies listed on the CMS Fact Sheet[12] as examples of formularies providing broad coverage for prescriptions drugs and biologicals, only one, Mass Health, listed any vaccines. Vaccines no doubt are covered under these health plans as part of the plan's medical benefit, but they do not appear on the formulary. Part D plans may have less of an incentive to include such therapies because the cost benefits of doing so are likely to be realized by other components of the Medicare program – e.g., Part A, to the extent that hospitalization is avoided as a result of vaccination, or Part B, where physicians services are avoided. We ask CMS to be aware of the unique nature of the Part D benefit when relying on private plan formularies as benchmarks and include a review of therapies that private plans may cover under the medical benefit that Medicare may more appropriately cover under Part D.

More appropriate benchmarks may be state programs that provide prescription cost assistance to low-income elderly residents. Model programs include New York's Elderly Pharmaceutical Insurance Coverage Program ("EPIC") and New Jersey's Pharmaceutical Assistance to the Aged and Disabled Program ("PAAD"). These programs provide assistance on a wide range of drugs and biologicals used by the elderly and are more likely to reflect the varying needs of this population.

[12] Kaiser, FirstHealth, AdvanceRx, MassHealth, Florida Medicaid. Note that the Kaiser weblink is not operational, and thus we are not able to verify whether Kaiser covers vaccines as part of the prescription drug benefit or as part of the medical benefit.

We urge CMS to clarify that all therapies currently covered under Medicare's replacement drug demonstration project should be covered on Part D formularies to ensure continuity of care for enrollees. The need for these therapies for the Medicare population already has been clearly established. We also wish to emphasize that this Part D coverage does not preclude coverage for the therapies under Part B.

Finally, we reiterate our comments on the Part D Proposed Rule regarding the coordination of benefits under Medicare Parts B and D. In reviewing formulary drug lists, we urge CMS to acknowledge that some drugs and biologicals may be covered appropriately under both Part B and Part D, depending on the medical judgment of the prescriber and the route of administration, and that formularies should reflect this possibility. As CMS has explained in the Proposed Rule, some covered Part D drugs could qualify for payment under Part B in some circumstances and Part D in other circumstances, depending on the route of administration and the way in which those drugs and biologicals are administered or dispensed. These determinations are to be made on an individual basis, and not with respect to coverage of a drug or biological as a whole.[13] In order to make self-administration a meaningful option for a subset of Part D enrollees able to do so, it will be important for plans to include these therapies on their formularies. We ask CMS to consider this aspect of plan formularies when conducting formulary reviews.

D. Widely Accepted Treatment Guidelines

BIO strongly supports CMS' proposal to use widely accepted treatment guidelines to guide a determination of whether Part D plans provide appropriate access to drugs and biologicals for diseases and conditions such as asthma, diabetes, HIV/AIDS, and psychological disorders. We believe that this approach is a critical component of ensuring that a plan design is not discriminatory and will help to evaluate whether a formulary includes the full range of medically necessary treatments for enrollees with certain conditions. As CMS has acknowledged, enrollees with chronic diseases such as AIDS will be "negatively impacted if they do not have access to a wide range of drugs in certain therapeutic classes and categories."[14] CMS has provided a partial list of diseases and disorders for which it may consider widely accepted treatment guidelines and

[13] *See* SSA § 1860D-2(e)(2)(B).
[14] 69 Fed.Reg. 46632, 46661 (Aug. 3, 2004).

notes that in some cases these treatment guidelines – along with widespread industry practices – require all or substantially all drugs and biologicals in a particular class to be covered.[15]

BIO asks CMS to consider widely accepted treatment guidelines for a broad range of diseases and disorders, not just those mentioned in the partial list CMS provides in the Draft Guidelines. Treatment of numerous diseases – such as multiple sclerosis, cancer, and ESRD – require access to a broad range of therapies. Formularies that fail to take into account widely accepted treatment guidelines for these and other diseases are likely to discriminate against enrollees with these diseases. We also ask that CMS be aware that even widely accepted treatment guidelines may not always reflect new treatments, and that any use of such guidelines should be in conjunction with consideration of newly available therapies and recent best practices. BIO urges CMS to recognize that treatment guidelines may be developed through an evidence-based review or by a consensus of clinical experts. The agency should consider the most authoritative guidelines, whenever available. Below we have listed some widely accepted treatment guidelines that BIO urges CMS to include in its formulary review:

- Cancer: NCCN/ACS Treatment Guidelines for Patients. National Comprehensive Cancer Network, 2004. http://www.nccn.org/professionals/physician_gls/default.asp; American College of Clinical Oncology, http://www.asco.org/ac/1,1003,_12-002009,00.asp.
- Cutaneous T Cell Lymphoma: Treatment of Cutaneous T Cell Lymphoma: Current Status and Future Directions.
- Diabetes: Diabetes Care, "Clinical Practice Recommendations" http://care.diabetesjournals.org/content/vol27/suppl_1/
- ESRD: The National Kidney Foundation, Kidney Disease Outcomes Quality Initiative (K/DOQI) www.kdoqi.org;
- Epilepsy: American Academy of Neurology, "Efficacy and Tolerability of New Antiepilectic Drugs 1: Treatment of New Onset Epilepsy, http://aan.com/professionals/practice/pdfs/clinician_ep_onset_e.pdf; Efficacy and tolerability of the new antiepileptic drugs II: Treatment of refractory epilepsy. Neurology 2004; 62(8):1252-73, http://aan.com/professionals/practice/pdfd/clinician_ep_refractory_e.pdf;

[15] Draft Guidelines at 9.

http://aan.com/professionals/practice/pdfd/clinician_ep_treatment_e.pdf. *See also* http://aan.com/professionals/practice/pdfs/patient_ep_onset_c.pdf; http://aan.com/professionals/practice/pdfs/patient_ep_onset_c.pdf; http://aan.com/professionals/practice/pdfs/patient_ep_refract_c.pdf; http://aan.com/professionals/practice/pdfs/patient_ep_treatment_b.pdf

- HIV/AIDS: DHHS, Panel on Clinical Practices for Treatment of HIV Infection, "Guidelines for the Use of Antiretroviral Agents in HIV-1-Infected Adults and Adolescents, (March 23, 2004);
- Mental Illness: American Psychiatric Association Practice Guidelines, www.psych.org/clin_res/prac_guide.cfm; Expert Consensus Guidelines Series, www.psychguides.com; Schizophrenia Patient Outcomes Research Team Treatment Recommendations, www.ahcpr.gov/clinic/schzrec.htm.
- Multiple Sclerosis: "Disease Management Consensus Statement" from the Medical Advisory Board of the National Multiple Sclerosis Society;
- Pain Management: Principles of Analgesic Use in the Treatment of Acute Pain and Cancer Pain, Fifth Edition, American Pain Society, 2003, http://www.ampainsoc.org/pub/principles.htm.
- Rheumatoid Arthritis: American College of Rheumatology (ACR) www.rheumatology.org/publications/guidelines/raguidelines02.asp
- Vaccines: Advisory Committee on Immunization Practices, http://www.cdc.gov/nip/publications/acip-list.htm.

E. Two Drugs or Biologicals Per Class

BIO appreciates CMS' recognition in the Draft Guidelines that two drugs or biologicals per class is a minimum requirement, but that more than two drugs or biologicals per class may be required "where additional drugs present unique and important therapeutic advantages."[16] We agree with CMS that – depending on the classification structure and the level of granularity – for many categories and classes, this minimum threshold will not be adequate to meet the statutory requirement that a plan design not discourage enrollment for certain groups of Medicare beneficiaries. A formulary that does not include well over two drugs or biologicals in many categories or classes may fail to provide a meaningful and comprehensive prescription drug benefit to Medicare beneficiaries.

[16] Draft Guidelines at 8.

For example, a plan that includes only two drugs in the classes within the antineoplastics category will necessarily be discriminating against individuals with certain types of cancer.[17] Cancer treatment is complex, and the types of agents used continue to evolve. Antineoplastics may be used for more than one organ system, for more than one type of cancer, for different stages of diseases, and often in combination with other agents. Cancer treatments also are not generally interchangeable and include antiemetics and treatments for anemia, neutropenia, and thrombocytopenia. Thus, it will be important that CMS review the drugs and biologicals available under a plan's formulary for beneficiaries needing cancer treatment in order to ensure that a beneficiary has appropriate access to necessary treatment. Similarly, enrollees with ESRD are a medically fragile population with very specific therapy needs; this population needs access to a wide range of therapies on an unrestricted basis. Plans certainly will need to provide more than two drugs or biologicals in a class to ensure appropriate access and meet the statutory requirement that a plan design not discourage the enrollment of ESRD patients.

F. Tier Placement

BIO appreciates CMS recognition that simply including a range of drugs and biologicals on a plan formulary will not be sufficient for CMS to approve the formulary as adequate. CMS states that it will "review tier placement to provide an assurance that the formulary is non-discriminatory."[18] Although we support CMS' intention to consider tier placement in evaluating and approving plan formularies, we are very concerned that the Draft Guidelines do not place adequate emphasis on review of cost containment strategies, such as excessive cost-sharing requirements, prior authorization, step therapy, or other requirements that limit enrollee access to formulary drugs and biologicals. A formulary that includes a wide range of drugs and biologicals but imposes excessive cost sharing requirements on many of these therapies does not provide enrollees adequate access to medically necessary therapies. Furthermore, an evaluation of tier placement is a critical part of determining whether a formulary discriminates against certain groups of beneficiaries. We respectfully request that CMS clarify that review of tier placement will be a central component of the formulary review process.

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[17] Private plans tend to include well over two drugs and biologicals, where available, in these types of classes. *See, e.g.* Kaiser Permanente (Colorado Springs) Drug Formulary.
[18] *Id.* at 8.

G. Inclusion of One Drug or Biological from Each of USP's Categories, Classes and Subdivisions

CMS has requested comment on whether an appropriate benchmark for determining whether a formulary is adequate would be whether the formulary includes at least one drug from each of USP's recommended categories, classes and subdivisions.[19] Although we support a baseline requirement that formularies include drugs and biologicals from each of the USP categories, classes, and subdivisions, BIO is concerned that this alone will not be adequate to ensure that a Part D plan is providing a comprehensive and nondiscriminatory prescription drug benefit. As we have stated in our comments to the Draft Model Guidelines and to the Proposed Rule, the Draft Model Guidelines fail to provide a classification system that will result in an adequate benefit.

We have not yet had the opportunity to review the final Model Guidelines, and thus we do not know whether USP has made the revisions necessary to ensure that the Model Guidelines will provide more appropriate categories, classes, and subdivisions than those in the Draft Model Guidelines. Unless the Model Guidelines have been dramatically improved to include the range of categories and classes necessary to ensure appropriate access to medically necessary therapies, an approval process based on whether *one* drug or biological from each category, class, and subdivision is listed on a Part D formulary will not provide adequate access.

For example, such an evaluation of a formulary would fail to ensure that the formulary include an appropriate range of vaccines. The USP Draft Model Guidelines include vaccines only as a recommended subdivision. This placement – combined with a formulary approval process that included a simple check of whether one drug or biological per subdivision is included on the formulary's drug list – would allow a Part D plan to cover only one vaccine. Vaccines target a range of diseases in the aged and disabled Medicare population and provide important wellness benefits to enrollees. Because vaccine use also is likely to be cost-efficient for the Medicare program, it would not make sense from the perspective of the Medicare program to approve a formulary that includes only one vaccine.

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[19] *Id.* at 9.

Similarly, such an evaluation could result in approval of a formulary that does not include drugs or biologicals used to treat rare diseases. For example, the Draft Model Guidelines establish "Enzyme Replacements/Modifiers" as a therapeutic category without any classes or subdivisions. This is despite the fact that each disease in this category is a rare disease caused by a unique deficiency or problem, and therefore therapies are not interchangeable. Because the Draft Model Guidelines do not account for these types of diseases and conditions, there is a significant risk that these therapies will not be included on a formulary if this approach to formulary approval is implemented. The loss of access to these treatments by enrollees would prove disastrous, particularly in the case of orphan drugs and biologicals, as these therapies often are the only viable therapy for Medicare beneficiaries and are not interchangeable with other therapies.

III. BENEFIT MANAGEMENT TOOLS

Prior Authorization, Step Therapy, and Generic Substitution

BIO appreciates CMS' recognition that cost containment tools – such as prior authorization, step therapy, or other mechanisms that restrict enrollee access to necessary therapies – are part of a plan's formulary and subject to CMS approval. We are concerned, however, that the Draft Guidelines do not propose sufficiently specific and stringent guidelines for evaluating plan use of these tools. In particular, as explained previously, we are concerned that CMS has not required that the minimum two drug or biologicals per category and class be available on an unrestricted basis (e.g., not subject to cost containment tools). Enrollees do not genuinely have access to therapies that are subject to such restrictions. We believe that the two drug or biological minimum requirement is intended as a means of ensuring enrollee access to an absolute minimum number of drugs and biologicals, and this requirement cannot be met by limited access to this minimum number of required drugs and biologicals. We ask CMS to clarify that at least two drugs or biologicals in each class must be available on an unrestricted basis in order to meet this formulary requirement.

We are particularly concerned that, absent more specific requirements, plans may subject many therapies to prior authorization and then fail to establish a prior authorization process that provides enrollees with genuine access to medically necessary therapies. For example, in our experience, some state Medicaid programs have attempted to restrict access to drugs and biologicals by

imposing prior authorization requirements and then understaffing telephone lines so that it is virtually impossible for beneficiaries or their physicians to actually obtain prior authorization. A critical part of providing beneficiaries meaningful access to drugs and biologicals subject to prior authorization is ensuring that plans have efficient prior authorization processes and respond promptly to beneficiary requests. P&T committees can help ensure that these processes are established in a manner that appropriately reflects the medical needs of a plan's enrollees and that benefit management tools such as step therapy requirements are instituted only where clinically appropriate. In considering what constitutes adequate access to therapies for which an enrollee must seek prior authorization or an exception to a step therapy requirement, we ask CMS to look to private plans that have succeeded in providing meaningful access. In particular, plans should not be permitted to impose step therapy that requires an enrollee to use a Part B or over-the-counter therapy before accessing a Part D drug or biological.

Similarly, it will be critical that a Part D enrollee receive a prompt response to an exceptions request. State Medicaid programs are required to respond to such requests within 24 hours of the receipt of a request.[20] Private plans typically follow similar timeframes, as discussed above.[21] We request that CMS establish clear standards in both the final Part D rule and the final formulary guidelines for plans that impose cost containment mechanisms such as prior authorization or step therapy. Without clear requirements, we are concerned that plans will not have an adequate incentive to ensure that benefit management tools do not hinder timely access to medically necessary therapies.

IV. CONCLUSION

BIO appreciates the opportunity to comment on the important issues raised in the Draft Guidelines, and we look forward to working with CMS to ensure that Part D formularies provide enrollees with appropriate access to a meaningful prescription drug benefit. In sum, BIO supports CMS' efforts to review the formularies of Part D plans using private sector benchmarks and knowledge of the specific needs of the Medicare population. Specifically, we support:

[20] Social Security Act § 1927(d).
[21] *See* note 8.

- The proposals on P&T committee membership requirements, quarterly meetings, and documentation of decisions in writing;
- The proposal that P&T committees must review each new chemical entity within 90 days of its release on the market or provide clinical justification for failure to do so;
- CMS' recognition that two drugs or biologicals per class is only a minimum threshold and may not provide adequate coverage for many diseases;
- CMS' intention to rely on widely accepted, current treatment guidelines to determine appropriate inclusion of therapies for diseases and conditions, including those listed by CMS as well as the full spectrum of uncommon diseases and disorder, in addition to relatively common illnesses such as cancer and other diseases.

Beyond our support for CMS' basic approach, we request clarification on the following issues raised in the Draft Guidelines:

- Require P&T committees to *approve* a plan's use of cost containment mechanisms that have clinical implications, such as prior authorization or step therapy;
- Clarify that P&T committees and plans may not reduce the number of classes or eliminate specific classes without CMS approval;
- Clarify the process for monitoring formularies on an ongoing basis;
- Establish clear and accessible procedures – including patient-based timeframes and the provision for the emergency supply of medication – in the final rule for plans to follow in considering exceptions requests;
- Limit exemptions from a review of a formulary's classification system to plans utilizing the USP Model Guidelines, with the understanding that this exemption is only one part of an evaluation of whether a plan design is acceptable;
- Ensure that CMS' drug list review includes full consideration of the needs of enrollees with rare diseases and disorders and that benchmarks used for such reviews are not limited to the top 25-50 therapies used by Medicare beneficiaries;
- Clarify that CMS intends to fully review tier placement to ensure that cost-sharing and other restrictions do not result in discrimination against certain groups of beneficiaries;

- Do not rely solely on a check of whether a formulary includes one drug or biological from each USP category, class, and subdivision as a proxy for approving formularies;
- Clarify that a minimum of two drugs or biologicals per class must be available on an unrestricted basis; and
- Ensure that evaluation of benefit management tools includes careful consideration of a plan's process for considering exceptions to cost containment mechanisms such as prior authorization or step therapy.

We appreciate CMS' consideration of these comments and would welcome the opportunity to discuss these issues in depth. Please contact Jayson Slotnik at (202) 312-9273 if you have any questions regarding our comments.

Respectfully submitted by,

/S/

Michael Werner,
Chief of Policy