BIO
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

Charlene Frizzera, Acting Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Room 445-G
Hubert H. Humphrey Building
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: CMS-4138-IFC4 (Medicare Program; Medicare Advantage and Prescription Drug Programs MIPPA Drug Formulary and Protected Classes Policies)

Dear Acting Administrator Frizzera:

The Biotechnology Industry Organization (BIO) appreciates this opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS) interim final rule regarding the establishment of a process for the establishment of protected categories and classes, published in the Federal Register on January 16, 2009 (the "Rule"),[1] pursuant to the Medicare Improvements for Patients and Providers Act (MIPPA). 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 1,200 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. Many of the therapies developed by biotechnology companies target conditions that primarily affect seniors. BIO has been a strong supporter of the Medicare Part D prescription drug benefit and appreciates CMS's significant efforts to implement this program. We believe that the Part D benefit has helped increase patient access to critical therapies as well as ensure that patients will be able to receive and afford the treatments that best meet their needs. We continue to encourage CMS to focus on patient access in its ongoing implementation and refinement of this important program.

[1] 74 Fed. Reg. 2881 (Jan. 16, 2009).

BIO greatly appreciates CMS's work in developing the protected categories and classes since 2006 and strongly supports the codification of a process to make protected categories and classes a permanent feature of Part D formulary requirements. Many of the therapies developed by BIO members serve the needs of very sick and extremely vulnerable Medicare patients. As CMS has recognized, the needs of these beneficiaries require special attention under Part D. It is critical that beneficiaries with chronic diseases such as HIV and cancer have access to a wide range of drugs and biologicals in certain therapeutic categories and classes.

The therapies used to treat these diseases typically are not interchangeable. A plan that includes a limited number of therapies from the antineoplastics category, for example, will necessarily be discriminating against individuals with certain types of cancer. Cancer treatment is complex, and the types of agents used continue to evolve rapidly. 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. Thus, it has been critical for cancer patients that CMS has required all of these therapies be on a plan formulary, in order to ensure that the full range of these therapies be available to Medicare beneficiaries. The same is true for the other classes that CMS has recognized as protected since the beginning of the Part D benefit, and BIO welcomes the development of a new process for establishing protected categories and classes as a way to help ensure that Medicare's most vulnerable patients have access to the range of therapies they need. BIO therefore urges CMS to adopt the designation of the existing six protected classes as this group has already been reviewed and approved and is a stable part of the Part D program.

In implementing the new process established under MIPPA for developing these protected categories and classes, BIO respectfully requests that CMS:

- Define what constitutes "widely used treatment guidelines" and augment the use of such guidelines with review of compendia and medical journals in order to take into account the needs of patients with uncommon conditions as well as to ensure patient access to newer therapies.
- Describe how Prescription Drug Event data will be used in establishing the protected categories and classes and the inclusion of drugs and biologicals

within those categories and classes, as well as recognize the inherent limits of any claims data in determining future patient needs.

- Refrain from establishing any exceptions to the statutory requirement that *all* Part D drugs in the protected categories and classes be included on formulary until the process for establishing such exceptions, as required by MIPPA, is complete.
- Limit the utilization management tools that plan sponsors may impose on drugs and biologicals within the protected categories and classes.

We have discussed each of these comments in greater detail below.

I. Widely Used Treatment Guidelines

BIO requests that CMS clarify how the review of treatment guidelines will be conducted and how the agency or its contractor will determine which treatment guidelines are considered "widely used." In the preamble to the Rule, CMS describes the process by which it will develop the protected categories and classes. As part of the first level of this process, CMS will engage a contractor that will "review all the widely used treatment guidelines and generate a list highlighting those categories or classes in which multiple drugs within classes or categories are typically used to treat a specific disorder."[2] Although BIO recognizes the valuable role that treatment guidelines play in synthesizing information for physicians and supports the establishment of a systematic method for reviewing potential protected categories and classes, we note that the prevalence of treatment guidelines may make this approach somewhat cumbersome. For example, the National Guideline Clearinghouse website currently lists 197 guidelines for mental disorders, 369 guidelines for AIDS, and 403 guidelines for neoplasms.[3] As one might expect from the sheer number of treatment guidelines available, such guidelines vary widely in their methodological rigor and protections from bias, and they often conflict.[4]

While treatment guidelines can provide valuable advice to physicians, they often are not current and typically do not contain information on the most recent treatments available. In fact, it may take years for a new therapy to be

[2] Id. at 2883.
[3] See National Guideline Clearinghouse at www.guideline.gov.
[4] See Institute of Medicine, Knowing What Works in Health Care: A Road Map for the Nation, at Ch. 5.

incorporated into treatment guidelines. BIO encourages CMS to rely on compendia and medical journal articles to address instances where treatment guidelines do not exist or are not adequate, such as where there are new treatments that have yet to be incorporated into the guidelines. Failure to incorporate newer therapies could leave patients without access to critical, life-saving therapies that may be the latest in the standard of care but not yet incorporated into treatment guidelines. Both compendia and peer reviewed journal articles reflect the cutting edge of care that may make the difference for patients as they battle cancer and other life-threatening diseases. Indeed, Congress recognized the importance of both of these types of publications when it decided to mandate coverage for certain cancer therapies included in them.

BIO also is concerned that reliance on "widely used" treatment guidelines could fail to ensure that enrollees with rare diseases or disorders have access to medically appropriate therapies. A significant percentage of biological therapies on the market were developed to treat rare diseases and disorders. An emphasis on widely used treatment guidelines may not capture less common conditions where access to multiple drugs and biologicals in a therapeutic category or class is important for medically appropriate treatment of these conditions.

BIO believes that CMS may improve the transparency of the process for identifying "widely used treatment guidelines" by relying more heavily on guidelines with certain desirable characteristics, similar to those utilized in the compendia process. These characteristics include: quick throughput including new evidence to update guidelines, a publicly transparent process for evaluating guidelines, public identification of the members of advisory/scientific review committees with broad representation to mitigate any perceived conflicts of interest, and a description of the evidence reviewed for each recommendation. By incorporating these elements into the evaluation of a treatment guideline, CMS may mitigate some of the concerns surrounding the guidelines that we note earlier in our comments.

Finally, BIO notes that guidelines are intended to help patients and providers make decisions and should not displace independent medical judgment. It is critical that physicians have available to them a broad range of therapies in order to appropriately treat patients with chronic and complex diseases, such as those for which protected categories and classes may be created. We encourage CMS to utilize treatment guidelines, compendia, and medical journals together to

develop the protected categories and classes and to ensure that physicians and patients have access to the full range of therapies in the categories and classes for which there is significant clinical need for access to a wide range of drugs and biologicals.

II. Prescription Drug Event Data

In the preamble to the Rule, CMS explains that it will provide information to its contractor on beneficiary utilization of multiple drugs within categories and classes based on analysis of prescription drug event (PDE) data.[5] BIO urges CMS to provide greater detail in further guidance on how this PDE data will be used to develop the newly established protected categories and classes and evaluate appropriate population of drugs and biologicals within these categories and classes to ensure that patients have access to the full range of medically necessary treatments.

In particular, BIO asks that CMS be cognizant of the limits of PDE data, or any claims data, in creating or populating the protected categories and classes. For example, claims data, if used to evaluate the frequency with which certain therapies are used, may not accurately reflect the medical necessity of therapies needed by certain subpopulations. Logically, this data also will not adequately capture therapies that have not been prevalent on Part D formularies in the past but may be necessary to adequately populate newly developed protected categories and classes. Past claims data also may overlook the existence and importance of innovative drugs that are new to the market and that, by extension, are not yet widely used. BIO encourages CMS to explain further how it will use the PDE data and to clarify that this data will not be used in isolation or without recognition of its inherent limitations.

III. Exceptions to Protected Classes

BIO urges CMS to follow the process required by MIPPA carefully when establishing any exceptions to drugs and biologicals covered within the protected categories and classes. Under MIPPA, Part D plan sponsors are required to include on formulary all Part D drugs in the designated protected categories and classes unless CMS establishes specific exceptions that allow plan sponsors to either exclude certain drugs or to place utilization management limits, such as prior

[5] 74 Fed. Reg. at 2883.

authorization, on any drugs.[6] Any such exceptions must be developed under a process that ensures that the coverage exception is based on scientific evidence and medical standards of practice. These exceptions also must be developed pursuant to a public notice and comment period.

In the preamble to the Rule, CMS appears to be trying to establish exceptions to the MIPPA requirement that "all part D drugs"[7] in the protected categories and classes be covered by stating that plan sponsors do not need to include all brand-name drugs and generic versions of drugs.[8] CMS states that it is the agency's longstanding policy that therapeutically equivalent drugs are considered the same drug for purposes of Part D formularies and then reaches the conclusion that this means that drugs that are not chemically distinct are not required to be placed in the protected categories and classes.[9]

BIO certainly supports CMS's efforts to ensure that Part D formularies are robust by clarifying that the threshold requirement that a formulary include two drugs per therapeutic category or class may not be met with two therapeutically equivalent drugs. Nonetheless, this threshold formulary requirement does not override the MIPPA statutory requirement that any exceptions to coverage of drugs in the protected categories and classes must be based on scientific evidence and medical standards of practice, as well as be subject to a public notice and comment process. As the statute contemplates, such an exceptions process must be related to the categories and classes that have been established. In the absence of categories and classes designated pursuant to the MIPPA process, it is impossible to know whether a blanket rule exempting coverage of drugs that are not chemically distinct is scientifically sound as applied to the specific categories and classes.

There may indeed be situations in which treatment guidelines, compendia, and medical journals will suggest that coverage of multiple drugs with the same active ingredient are appropriate. For example, CMS and its contractor may consider coverage of extended release therapies where an immediate-release therapy also is on formulary. Sometimes, it is incremental innovation that makes a significant difference in the lives of patients, such as the development of extended

[6] Social Security Act § 1860D-4(b)(3)(G)(iii).
[7] Id. § 1860D-4(b)(3)(G)(ii).
[8] 74 Fed. Reg. at 2883.
[9] Id.

release formulations of existing therapies. In other classes, drugs with the same active ingredient, but with different inactive ingredients, may be tolerated differently by different patients. Consistent with MIPPA, BIO urges CMS to establish any exceptions to the inclusion of all drugs and biologicals in a protected category or class only when warranted by scientific evidence and medical standards of practice, and only after a notice and comment period in which the public has the opportunity to provide input on the protected categories and classes and the applicable exceptions.

IV. Prior Authorization or Other Utilization Controls

BIO urges CMS to permit only the most limited utilization management tools, if any, on drugs and biologicals in the newly established protected categories and classes. BIO supports CMS' existing policy restricting the use of utilization management tools on HIV/AIDS drugs, with a very limited exception. We urge CMS to extend this approach to the drugs and biologicals within each of the newly established protected classes. As CMS has stated, the agency instituted its policy of establishing protected classes in order to ensure that "Medicare beneficiaries reliant upon these drugs would not be substantially discouraged from enrolling in certain Part D plans, as well as to mitigate the risks and complications associated with any interruption of therapy for these vulnerable populations."[10] Indeed, in past years, CMS stated its expectation that, for patients already stabilized on a drug, "plans would not use management techniques like prior authorization or step therapy, *unless a plan can demonstrate extraordinary circumstances.*"[11] BIO appreciates Congress' recognition of the importance of broad access to drugs and biologicals in the categories and classes designated as protected, as suggested by the requirement that any limitations on the formulary inclusion of all Part D drugs in these categories and classes follow the exceptions process required by MIPPA and be based on scientific evidence and standard medical practice, as well as subject to a notice and comment process.

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[10] CMS, Prescription Drug Benefit Manual, Ch. 6, §30.2.5.

[11] CMS Guidance, "Why is CMS requiring 'all or substantially all' of the drugs in the antidepressant, antipsychotic, anticonvulsant, anticancer, immunosuppressant and HIV/AIDS categories, posted at http://www.cms.hhs.gov/PrescriptionDrugCovContra/Downloads/FormularyGuidanceAllorSubAll.pdf (emphasis added).

BIO understands the importance of utilization management in the broader context of the prescription drug benefit and is aware of its importance to the continued viability of the Part D program. BIO hopes to continue to work with CMS to ensure that utilization management of other non-protected drug classes is appropriate and that patients continue to have access to treatment.

V. Conclusion

BIO appreciates the opportunity to comment on this Rule. We look forward to continuing to work with CMS to address these critical issues in the future. Please feel free to contact Laurel Todd at 202-962-9220 if you have any questions or if we can be of further assistance. Thank you for your attention to this very important matter.

Respectfully submitted,

/s/

Laurel Todd
Director, Reimbursement
& Economic Policy