October 15, 2018

The Honorable Alex Azar
Secretary
U.S. Department of Health & Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: Step Therapy for Part B Drugs in Medicare Advantage (MA)

Dear Secretary Azar,

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprised of more than 230 institutions and individuals across the health care spectrum, is writing to express concern with a recent decision made by the Centers for Medicare & Medicaid Services (CMS) to allow Medicare Advantage (MA) plans to utilize step therapy for Medicare Part B drugs as a tool for containing costs. We believe that this reversal in long-standing policy will negatively impact progress made in delivering personalized medicine to patients. CMS can address this concern by suspending the new policy until all stakeholders are consulted and agree to a path forward that protects the patient-physician relationship and fosters the continued advancement of personalized medicine.

Personalized medicine is an evolving field that uses diagnostic tools to identify specific biological markers, often genetic, to help determine which medical treatments and procedures will be best for each patient. By combining this information with an individual's medical history, circumstances, and values, personalized medicine allows doctors and patients to develop targeted prevention and treatment plans.

Personalized medicine is helping to shift the patient and provider experience away from trial-and-error and toward a more streamlined process for making clinical decisions, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction. PMC's members are leading the way in personalized medicine and recommend that patients who may benefit from this approach undergo appropriate testing and tailored treatment as soon as possible during their clinical experiences.

PMC recognizes the important role that utilization management tools like step therapy play in helping health insurance companies manage health care costs. It is vital, however, that these tools support clinically appropriate care and align with the rapidly emerging science and practice of personalized medicine. Recent research points to the significant promise of innovative decision-support platforms to improve patient care and reduce total treatment costs. We urge CMS to advance policy that harnesses next-generation decision-support and avoids reinforcing conventional policies that would blunt the adoption of personalized medicine.

Step therapy could halt progress in personalized medicine at a critical time.

In January, PMC published *Personalized Medicine at FDA: 2017 Progress Report*,[i] which demonstrates how personalized medicine has reshaped drug development over the last decade in ways that are benefiting patients. Our report documents a record number of new personalized medicine approvals by the U.S. Food and Drug Administration (FDA), including the first three gene therapies ever approved in the U.S. and the first personalized medicine biosimilar. This marks the fourth consecutive year that personalized medicines accounted for more than 20 percent of all new drug approvals.

Fortunately for patients, the pipeline for significant personalized therapies is robust. Many of the personalized medicines approved by the FDA and in the pipeline are Part B drugs targeted for patients with diseases and conditions that have very poor prognoses, such as advanced breast cancer, non-small cell lung cancer, leukemia, lymphoma, and cystic fibrosis, among others.

Advances in science made possible through significant investments from government as well as the biopharmaceutical and diagnostics industries have transformed once-deadly diseases into manageable chronic conditions for some patients. Medical product development is focusing more on therapeutics that define patient populations based on biological signatures and other factors that may influence an individual patient's response. In contrast, step therapy perpetuates a one-size-fits-all treatment paradigm that facilitates the same treatment protocol for every patient, regardless of their biological differences. Instituting step therapy in Part B can therefore rob patients of time and reduce their quality of life by pre-empting the use of personalized treatments that have a high likelihood of working for them.[ii]

Step therapy could delay patient access to optimal treatment and fail to produce cost savings.

Step therapy requires a patient to try a lower-cost treatment before working up to a more expensive product if the initial treatment is ineffective. Without clear safeguards in place, this approach could delay what health care providers deem to be the medically appropriate treatment for a patient at the most appropriate time. For diseases like cancer, modern treatment is individualized and lacks interchangeable treatment options. Requiring a patient to fail first on a less expensive treatment limits a provider's ability to prescribe a treatment based on the best available evidence and impacts a patient's ability to control his/her disease. We fear that this may lead to poorer outcomes that may not be reversible.

Personalized medicine is helping patients and providers understand the variability in patients' responses to treatments for cancer and other diseases, enabling the effective, efficient targeting of the best treatment regimen for each patient. Conventional step therapy policies, in contrast, are based on judgments of treatments' relative clinical value across a broad population, creating barriers to tailored treatment regiments and chilling adoption of personalized medicine.

Many Part B drugs are intended for patients with cancer and autoimmune diseases.[iii] In severe cases of an autoimmune disease like rheumatoid arthritis and advanced cancer, time is of the essence and step therapy can undermine attempts to treat them before they get worse. Using clinical judgement, health care providers have been able to keep many patients out of hospitals and avoid unnecessary toxicities because they currently have the ability to choose the most effective drug. If they are placed in the position of having to try medications that are known not to work for a specific patient, patients will be at risk for side effects that may require emergency room visits or hospital admissions. A 2010 study showed that although step therapy can reduce insurers' short-term costs, it can hinder patient health and indirectly cause increased long-term costs.[iv]

Statement of Neutrality

Many of PMC's members will present their own responses to the CMS decision and will actively advocate for those positions. PMC's comments are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments and positions.

Conclusion

We recognize the increasing pressure to contain overall health care costs, but PMC urges the Administration to focus on solutions that bring us closer to the goal we share with you of delivering appropriate, efficient, and accessible health care to every patient. PMC welcomes the opportunity to serve as a resource for you. If you have any questions about the content of this letter, please contact me at 202-589-1769 or cbens@personalizedmedicinecoalition.org.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

CC: Seema Verma, Administrator, Centers for Medicare & Medicaid Services

[i] Personalized Medicine Coalition. *Personalized Medicine at FDA 2017 Progress Report*. January 2018. Accessed October 12, 2018 at http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_2017_Progress_Report.pdf

[ii] Harvard Business Review. *Realizing the Promise of Personalized Medicine*. October 2007. Accessed July 12, 2018 at https://hbr.org/2007/10/realizing-the-promise-of-personalized-medicine

[iii] Xcenda Amerisource Bergen. *Medicare Physician-Administered Drugs: Do Providers Choose Treatment Based on Payment Amount?* September 19, 2018. Accessed October 12, 2018 at https://www.xcenda.com/-/media/assets/xcenda/english/content-assets/white-papers-issue-briefs-studies-pdf/xcenda_partb_provider-utilization.pdf?la=en&hash=5C05816DFD9F8952AE5E5076EF2A40565507BCCC

[iv] Carlton, R.I., Bramley, T.J., Nightengale, B., Conner, T.M., Zacker, C. *Review of Outcomes Associated with Formulary Restrictions: Focus on Step Therapy*. April 6, 2010. Accessed October 12, 2018 at https://www.ajpb.com/journals/ajpb/2010/vol2_no1/review-of-outcomes-associated-with-formulary-restrictions-focus-on-step-therapy