PERSONALIZED MEDICINE COALITION

March 30, 2016

The Honorable Joe Biden
Vice President of the United States
The White House
1600 Pennsylvania Ave., N.W.
Washington, D.C. 20500

Re: Request for Information from the Precision Medicine Community

Dear Mr. Vice President:

Thank you for engaging the precision medicine community as Chair of the Cancer Moonshot Task Force and for calling on families, researchers and physicians across the country to join the effort. Your focus on making the most of federal investments, targeted incentives, private sector efforts from industry and philanthropy, patient engagement initiatives, and other mechanisms to support cancer research and enable progress in treatment and care will help accelerate the pace of medical breakthroughs in oncology. These investments have the potential to greatly improve the quality of patient care in the United States and promote personalized medicine, which is on the cutting edge of biomedical innovation.

This letter is in response to the one you sent the attendees of the White House's Precision Medicine Initiative Summit on February 25th 2016, in which you invited feedback on how to seize this incredible moment that offers hope for patients and families and that defines America's unrelenting pursuit of discovery.

As you indicated in your letter, the concept of personalized medicine presents challenges to the current system and to health care policymakers. The core of these challenges is the fact that conventional policy has treated therapeutic agents, diagnostic tests, and health care services as separate policy issues, while personalized medicine requires that policies governing these different health care segments be aligned.

The Personalized Medicine Coalition (PMC) is an education and advocacy organization that represents innovators, scientists, patients, providers and payers to promote the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

As you know, personalized medicine is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient. By combining the data from those tests with an individual's medical history, circumstances and values, health care providers can develop targeted treatment and prevention plans. The goal is to provide the right treatment to the right patient at the right time. The pace of progress for the evolving field is accelerating rapidly.

Of the 45 novel new drugs (NNDs) approved by the Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER) in 2015, 13 of them — more than 25 percent — were personalized medicines as classified by PMC, thus continuing a trend that began in 2014 when PMC classified nine of 41 NNDs as personalized medicines. As a point of comparison, there were only 13 total personalized medicines on the market in 2006. Today there are more than 150.

Personalized Medicine Coalition
personalizedmedicinecoalition.org
1710 Rhode Island Ave., NW, Suite 700
Washington, DC 20036
P: 202.589.1770
F: 202.589.1778

Nowhere is the transformation of health care toward personalized medicine more clear than in oncology. Of the 13 personalized NNDs in 2015, five are oncology drugs. These drugs account for 35 percent of the 14 oncology NNDs approved in 2015. The high proportion of new approvals that are personalized medicines demonstrates the progress researchers have made in advancing the field from an emerging idea a decade ago to an established approach to treating many diseases today.

With that in mind, PMC has identified a number of suggestions in the areas of regulatory policy, reimbursement and payment policies, and public and private sector research that we believe the National Cancer Moonshot effort could incorporate to achieve maximum impact. Each of these suggestions would accelerate progress in oncology through personalized medicine.

POLICY SUGGESTIONS

Regulatory Policy:
Incentivize personalized medicine by creating a transparent, stable, and predictable regulatory environment for personalized medicine products that is flexible enough to respond to the emerging science.

Traditional methods of oversight may be obsolete for the dynamic tools that make personalized medicine possible. This presents both challenges and opportunities.

Regulatory agencies have an opportunity to design new approaches that can provide meaningful oversight of breakthrough therapies and novel technical diagnostic platforms with dynamic clinical evaluative capabilities. While considering new oversight processes, it is important to work with all stakeholders to develop important details on how those processes would be resourced, standardized and administered. Future investment and technological advancement in personalized medicine depends on clear, predictable guidelines. Oversight approaches need to appropriately foster innovation and allow timely access to new personalized medicine information while ensuring accuracy and reliability. Improperly constructed regulatory processes might cause unintended consequences, such as reduced patient access to personalized medicine products that improve health outcomes.

Reimbursement and Payment Policies:
Assure that coverage and reimbursement policies support continued innovation and adoption of personalized medicine.

Coverage and payment policies could be transformed to align with the principles of personalized medicine and ensure that these advances reach patients and improve health care outcomes. Federal policies should encourage the use of personalized medicines, which can drastically improve the quality of care patients experience, extend life, reduce morbidity and bring efficiencies to the health system.

The Centers for Medicare and Medicaid Services (CMS) are currently exploring alternatives to the traditional fee-for-service model historically used to pay for health care services. If improperly designed, these programs could negatively impact the field. CMS could be charged with designing new models that support personalized medicine. For example, under current law, whether Medicare provides coverage for a particular drug/biologic is independent of whether it provides coverage for a particular diagnostic, and vice versa. Medicare statute should be altered to provide coverage of a personalized medicine diagnostic test that is prescribed, recommended, referenced, or suggested for use in the FDA-approved labeling of a personalized medicine drug/biologic for which Medicare coverage is available. CMS should take care to ensure that its policies do not unintentionally impede the continued development of personalized medicine and the associated advances it offers patients.

Public-Private Partnerships:
Encourage research by the public and private sectors as well as public-private partnerships.

Sustaining advances in personalized care and treatment will require the combined resources of multiple stakeholders — all of whom must be willing to invest in a paradigm change that can preserve innovation, improve outcomes and reduce

the overall costs of health care. We appreciate the high level of engagement the administration is having with research communities in the public and private sectors, and encourage the administration to continue that engagement.

We hope you will find the following educational materials useful to the National Cancer Moonshot initiative.

EDUCATIONAL MATERIALS

2015 Progress Report: Personalized Medicine at FDA
Documents the upward trend in the number of personalized medicine approvals at FDA.
http://www.personalizedmedicinecoalition.org/Resources/2015_Progress_Report_Personalized_Medicine_at_FDA

The Case for Personalized Medicine
Outlines the current state of personalized medicine science, policy, and business. It is the go-to resource on this topic, and includes a table of personalized medicine products, real-world examples that demonstrate how personalized medicine is improving the quality of patient care and a discussion of policies impacting the field.
http://www.personalizedmedicinecoalition.org/Resources/The_Case_for_Personalized_Medicine

Personalized Medicine by the Numbers
Quantifies progress in the field.
http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_by_the_Numbers

Paying for Personalized Medicine: How Alternative Payment Models Could Help or Hinder the Field
Explores how alternative payment models may promote or impede access to personalized medicine tests and treatments.
http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/paying_for_personalized_medicine.pdf

Pathways for Oversight of Diagnostics
Outlines the laws and regulations that govern personalized medicine diagnostics so that all stakeholders can share a common understanding of the current system as they seek to define improvements to it.
http://www.personalizedmedicinecoalition.org/Resources/Pathways_for_Oversight_of_Diagnostics

CONCLUSION

PMC commends you on your work with the National Cancer Moonshot initiative and appreciates your outreach to the precision medicine community. We would welcome the opportunity to discuss these ideas in further detail during a meeting with you and your staff.

If you have any questions or require more information, please contact Amy M. Miller, Ph.D., by phone at 202-589-1769 or email at amiller@personalizedmedicinecoalition.org.

Sincerely yours,

Edward Abrahams

Edward Abrahams

President