

June 1, 2014

The Honorable Fred Upton (R-MI)
Chairman
House Energy & Commerce Committee
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Diana DeGette (D-CO)
Member
House Energy & Commerce Committee
2125 Rayburn House Office Building
Washington, D.C. 20515

Sent via e-mail: Cures@mail.house.gov

Re: Request for Information Regarding the 21st Century Cures Initiative

Dear Chairman Upton and Representative DeGette:

Thank you for engaging the community on the 21st Century Cures initiative. Your focus on accelerating the pace of medical breakthroughs should generate ideas and legislation that greatly improve the quality of patient care in the United States, including proposals to promote personalized medicine, which is on the cutting edge of biomedical innovation.

This letter is in response to your request for comments published in the white paper entitled *21st Century Cures: A Call to Action*.

The Personalized Medicine Coalition (PMC) is an education and advocacy organization that promotes the understanding and adoption of personalized medicine to benefit patients and the health care system. We represent more than 225 academic, patient, provider, and payer organizations, as well as drug and diagnostic manufacturers and clinical laboratories. Given the hopes and desires of this diverse group of stakeholders united in PMC, the Coalition has a keen interest in the 21st Century Cures initiative.

Personalized medicine uses diagnostic tools to identify specific biological markers, often genetic, that can help assess which medical treatments and procedures will work best for each patient. By combining this information with an individual's medical history and circumstances, personalized medicine allows doctors and patients to develop cost-saving, targeted prevention and treatment plans. Personalized medicine, therefore, has the potential to optimize the delivery and dosing of treatments so patients can receive the most benefit at the least amount of risk and harm, eliminating both the unnecessary side effects of toxic treatments such as chemotherapy and the delays associated with the "trial-and-error" process that many patients endure to obtain the correct diagnosis and treatment for their condition.

At a time of unprecedented scientific and medical breakthroughs, personalized medicine has the capacity to more accurately diagnose human diseases, predict individual susceptibility to disease, detect the onset of disease at early stages, pre-empt its progression, target treatments, and increase the overall efficiency and effectiveness of the health care system.

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These advances have already impacted the way we treat patients. Metastatic melanoma and certain types of lung cancer are now further classified by their molecular signatures, and are treated with the drug that is most likely to improve the patient's chance of survival based on that signature. The number of personalized medicine products has more than quadrupled in recent years, from only 13 on the market in 2006 to more than 70 in 2012. These innovations are changing the face of health care today, as researchers further investigate the heterogeneity of disease and work together to develop solutions that improve patient care and reduce overall health care costs.

But our current system is not capable of managing the new characterization of diseases. Many of our most costly and prevalent diseases are in fact a collection of different diseases. Soon we will be able to correctly categorize them as distinct entities, thereby aiding treatment, and ideally, contributing to the development of cures for diseases such as type-2 diabetes, schizophrenia, and cystic fibrosis. The change in how we characterize disease will require resources to retrain health care professionals, adopt new infrastructure, and identify new ways to inform patients so that they can better understand their condition.

Current progress in personalized medicine is a harbinger of much greater things to come. Patients with rare and devastating diseases can now lead normal and economically productive lives, and in doing so decrease the overall health care burden and dramatically increase their own quality of life. Previously devastating cancers can now be treated while patients remain productively employed, and children with certain genetic conditions can be diagnosed and treated early, enabling them to lead normal lives.

The concept of personalized medicine, however, presents challenges to health care policymakers. At the heart of these challenges is the fact that while conventional policy has treated therapeutic agents, diagnostic tests, and health care services as separate policy issues, personalized medicine requires that policies governing these different segments of health care be aligned.

POLICY SUGGESTIONS

As outlined in the white paper, many have recognized that to reap the rewards of our \$3 billion federal investment in mapping the human genome, comprehensive policy adjustments must be made. PMC has identified a number of concepts that would enhance patient health care delivery through personalized medicine.

PMC's advocacy activities focus on regulatory policy, reimbursement and payment policies, and incentives for biomedical innovation. In this letter, we briefly outline our suggestions in the areas where we think Congressional intervention could have maximum impact.

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.

This can be accomplished through coordinated review of personalized medicine products and concurrent review of qualified, co-developed analyte diagnostics, as augmentation to the current expedited review pathway for breakthrough therapies. Under current law, the timing of the FDA's review of a drug/biologic is unrelated to the timing of the agency's review of the test designed to guide its use.

Reimbursement and Payment Policies:

Assure that coverage and reimbursement policies support continued innovation and adoption of personalized medicine.

Federal payment policies should incentivize personalized medicine and accommodate personalized approaches to care, as opposed to basing decisions on average responses. CMS is currently exploring alternative models for

paying for health care. 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.

Incentives for Biomedical Innovation:

Encourage research by the public and private sectors as well as public-private partnerships.

This could be accomplished by supporting the National Institutes of Health (NIH) and the research and development credits that encourage innovation. The NIH has fueled the American biomedical innovation engine by educating young scientists and encouraging the science that supports quality improvements in health care. The availability of the Qualifying Therapeutic Discovery Tax Project tax credit/cash grant program, designed to encourage projects aimed at treatments for unmet medical needs and/or prevent, detect, or treat chronic or acute diseases and conditions, should be extended. Furthermore, the risks of co-developing therapeutic-diagnostic combinations could be reduced through the establishment of a new research and development tax credit that encourages the development of novel personalized medicines and their co-developed diagnostics.

We hope you will find the following educational materials useful to the 21st Century Cures initiative.

EDUCATIONAL MATERIALS

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. It 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](http://www.personalizedmedicinecoalition.org/Resources/The_Case_for_Personalized_Medicine)

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](http://www.personalizedmedicinecoalition.org/Resources/Pathways_for_Oversight_of_Diagnostics)

Personalized Medicine by the Numbers

Quantifies progress in the field.

[http://www.personalizedmedicinecoalition.org/Resources/Personalized Medicine by the Numbers](http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_by_the_Numbers)

The Future of Coverage and Payment for Personalized Medicine Diagnostics

Defines the personalized medicine reimbursement landscape 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/The Future of Coverage and Payment for Personalized Medicine Diagnostics](http://www.personalizedmedicinecoalition.org/Resources/The_Future_of_Coverage_and_Payment_for_Personalized_Medicine_Diagnostics)

CONCLUSION

PMC commends the House Energy and Commerce Committee's work and shares in your goal of charting the course for discovery, development, and delivery of health care advancement by promoting new policies that support innovation and the development of products and services that deliver high-quality, efficient, patient-centered care. We will expand on our policy suggestions in the coming months.

PMC appreciates the opportunity to provide comments on the 21st Century Cures initiative. If you have any questions about these comments or would like more details, please contact me at 202-589-1770 or via e-mail at amiller@personalizedmedicinecoalition.org.

Sincerely,

A handwritten signature in cursive script that reads "Amy M. Miller".

Amy M. Miller, Ph.D.
Executive Vice President
Personalized Medicine Coalition (PMC)