PERSONALIZED MEDICINE COALITION

December 16, 2019

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Fred Upton
U.S. House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

Sent electronically

Re: Cures 2.0 Request for Information

Dear Representative DeGette and Representative Upton:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 200 institutions across the health care spectrum, thanks you for releasing a request for information (RFI) on Cures 2.0.[i] The preceding *21st Century Cures Act* made meaningful regulatory changes and provided essential financial support for many of the breakthroughs in personalized medicine that patients are benefitting from today. We appreciate the opportunity to provide preliminary feedback on the scope and approach outlined in the RFI. Our comments are limited to the priority areas identified in the RFI and how Cures 2.0 can continue to support advances in 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 treatments of late-stage diseases in favor of more streamlined approaches to disease prevention and treatment, 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.

Experts have highlighted that personalized medicine is delivering better efficacy, improvements in overall survival, and a reduction in adverse events for patients.[ii] PMC is helping to build the evidence base necessary to further demonstrate the clinical and economic value of personalized medicine to inform policies that promote its widespread adoption.

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PMC has observed that the field continues to experience challenges delivering timely individualized care. Obstacles impeding the integration of personalized medicine are often caused when scientific developments outpace updates to our regulatory, payment, and health care delivery systems. In the current environment, patients, providers, and other health care stakeholders are not always prepared to make informed decisions about personalized medicine based on an assessment of all available diagnostic and treatment options. We hope that your emphasis on coverage issues, real-world evidence (RWE), and digital health in the Cures 2.0 RFI will inform thoughtful policy solutions that may alleviate some of these challenges as they relate to personalized medicine.

Statement of Neutrality

Many of PMC's members will present their own responses to the Cures 2.0 RFI. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance, and are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to this RFI or related issues.

Coverage and Reimbursement

The number of personalized treatments on the market, which PMC uses as a proxy for progress in personalized medicine, has increased dramatically since 2008, when there were five personalized medicines available. Today there are more than 170 personalized therapies on the market in the U.S.,[iii] a number that continues to grow as the Food and Drug Administration (FDA) rapidly approves more tests and treatments that can facilitate more effective and efficient health care based on the use of diagnostic tests that determine which medical treatments will work best for each patient.

To ensure that patients have access to personalized medicine, PMC advocates for flexible coverage policies and adequate payment rates for personalized medicine treatments and technologies. PMC has been working with the Centers for Medicare & Medicaid Services (CMS) and private payers to inform strategies that facilitate access to personalized medicine based on the value it provides to patients, the health care system, and society. We have been fortunate to see many transformative treatments and technologies come to market in recent years. CAR T-cell therapies in oncology, gene therapies for pediatric rare diseases, and next-generation sequencing technologies are just a few of the innovations that are unlocking a new era of personalized care. Unfortunately, the process for seeking and securing coverage for these technologies and treatments by CMS and private payers has been challenging. In some cases, inconsistencies in coverage and inadequate reimbursement have impacted patient access.

Questions remain about the level of evidence required to demonstrate clinical utility of many personalized medicine tests. Data required for offering a test or in seeking FDA approval for a test differ in type and in application from data needed to inform reimbursement decisions. The high costs of new personalized treatments, limited data on their performance beyond very specific patient populations, and variations in delivery methods put pressure on our reimbursement system, which was designed to pay for small molecule drugs and less complex biologic products based on data from randomized controlled clinical trials (RCTs).

Realizing the potential of personalized medicine will therefore require a paradigm shift in coverage and reimbursement. There is a need for closer discussions and clearer communications between drug and diagnostic developers, regulators, payers, health care providers, and patients earlier in the development process so that standards for evidence generation are clearer. Additionally, Congress should consider assisting the CMS Coverage and Analysis Group (CAG) in recruiting and retaining additional personnel. The CAG is small relative to the volume and complexity of national coverage determinations being pursued. The *21st Century Cures Act* emphasized FDA's need for more highly trained experts in specialized fields. We believe the same level of expertise is necessary for those individuals shaping national policies for coverage of novel treatments and technologies.

Real-World Evidence

The *21st Century Cures Act* placed an additional focus on the use of RWE to support regulatory decision-making, including approval of new indications for approved drugs. Congress defined RWE as data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than traditional clinical trials. FDA expanded on this definition and now defines RWE as clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of data routinely collected from electronic health records (EHRs), claims and billing sources, product and disease registries, patient-generated data, and mobile devices.[iv]

RWE is enabling researchers to go beyond the scope of traditional clinical trials and to gain insights from information collected in routine clinical care. However, using this data to tailor care at an individual patient level is complex because providers do not always have the resources to assess every piece of information that could help a patient achieve their specific health outcomes. Advanced tools like artificial intelligence, machine learning, and other analytics technologies are playing an increasingly important role in continually assessing records and other data sets to extract pertinent information.

Health care delivery informed by RWE is an important part of personalized medicine's future. PMC appreciates your desire to build on the initial RWE provisions in the *21st Century Cures Act*. We look forward to working with you as you identify legislative opportunities to support ongoing RWE activities at the FDA and develop new initiatives across the federal government to foster enabling technologies that will make data-driven health care a reality for more patients.

Digital Health

Digital health is an approach focused on using such technology to monitor and provide relevant health-related data about individuals. These technologies include a rapidly expanding array of consumer products and wearables, as well as complex clinical care platforms.[v] The collection of accurate digitized information that can be integrated with other data is essential to personalized medicine, and we are pleased to see it highlighted as a priority in the Cures 2.0 RFI.

A 2017 *New England Journal of Medicine Catalyst* article notes that fewer digital health products than expected are being deployed in real-world clinical settings.[vi] The authors attribute issues with deployment to a number of factors, one being the failure to improve quality and outcomes

and/or reduce costs in managing diseases with some digital health technologies. PMC believes that there needs to be a balance between activities to test and validate digital health products in controlled environments (e.g., clinical trials) and those that encourage evidence generation demonstrating the value of these technologies in daily practice. An emphasis on methods for not only examining the accuracy and reliability of digital health technologies, but also their clinical and economic benefit, is essential to ensure coverage and more widespread adoption. We hope Cures 2.0 can help support both of these needs to help the field advance.

Conclusion

Thank you for releasing the RFI and for considering our comments. PMC welcomes the opportunity to serve as a resource for you as you continue the Cures 2.0 effort to ensure it can support the ongoing development and delivery of personalized medicine products and services for all patients. If you have any questions about the content of this letter, please contact me at 202-589-1769 or cbens@personalizedmedicinecoalition.org.

Sincerely yours,

Cynthia A. Bens
Senior Vice President, Public Policy

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[i] Representative Diana DeGette and Representative Fred Upton. *Request for Information: Cures 2.0*. November 22, 2019. https://degette.house.gov/sites/degette.house.gov/files/Cures%202.0%20Call%20to%20Action%20Document.pdf.

[ii] Charles River Associates. *The Benefits of Personalized Medicine to Patients, Society and the Healthcare System: Final Report*. Prepared for the European Biopharmaceutical Enterprises and the European Federation of Pharmaceutical Industries and Associations. July 6, 2018. https://www.ebe-biopharma.eu/wp-content/uploads/2018/07/CRA-EBE-EFPIABenefits-of-PM-Final-Report-6-July-2018-STC.pdf.

[iii] Personalized Medicine Coalition. *Strategic Plan 2020*. December 2019. http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_Strategic_Plan_20201.pdf

[iv] U.S. Food and Drug Administration. *Real-World Evidence*. https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence. Accessed December 13, 2019.

[v] University of California, San Francisco. *The Elements of Personalized Medicine*. https://precisionmedicine.ucsf.edu/digital-health. Accessed December 13, 2019.

[vi] Kvedar, J.C., Fogel, A.L. "Why Real-World Results Are So Challenging for Digital Health". *New England Journal of Medicine Catalyst*. July 10, 2017. https://catalyst.nejm.org/doi/full/10.1056/CAT.17.0453.