



October 13, 2023

The Honorable Michael C. Burgess, M.D.  
Chair, Health Care Task Force  
House Budget Committee  
U.S. House of Representatives  
2161 Rayburn House Office Building  
Washington, DC 20515

**Re: Request for information on solutions to improve outcomes and reduce federal health care spending**

Dear Representative Burgess and distinguished members of the Health Care Task Force:

The Personalized Medicine Coalition (PMC), which represents more than 200 scientific, industry, patient, provider, and payer institutions to promote the understanding and adoption of personalized medicine concepts, services, and products for the benefit of patients and the health care system, thanks the House Budget Committee Health Care Task Force for the opportunity to provide information on examples of evidence-based, cost-effective health interventions that can reduce long-term health care costs, as well as opportunities for Congress to help address related regulatory and implementation barriers.<sup>1</sup>

In response to this request for information, PMC would like to call your attention to recent studies on pharmacogenomic (PGx) testing and diagnostic testing for rare diseases. With broader implementation, both PGx and genomics-based diagnostic testing have the potential to improve patient outcomes and reduce health care spending in the United States by directing the right treatments to the right patients, sooner, thus keeping American patients healthier, longer. With support from this Coalition, leaders of the Congressional Personalized Medicine Caucus have proposed the *Right Drug Dose Now Act of 2023*, the *Pharmacogenomics Research and Education Act*, and the *Precision Medicine Answers for Kids Today Act*, which together would encourage the broader utilization of diagnostic testing in both areas to improve outcomes and help United States health care providers make better use of finite resources. We encourage the House Budget Committee Health Care Task Force to help advance personalized medicine for the benefit of patients and health systems by co-sponsoring these bills.

PMC defines personalized medicine as an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history,

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circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients.

Personalized medicine is helping to shift the patient and provider experiences 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 and its members are leading the way in personalized medicine and in developing evidence showing how patients and the health care system can benefit from appropriate testing and tailored treatment as soon as possible during their clinical experiences.

### **Statement of Neutrality**

PMC's members may present their own responses to this request for information and actively advocate for those positions. PMC's response is designed to provide feedback so that the general concept of personalized medicine can advance, and is not intended to impact adversely the ability of individual PMC members, alone or in combination, to submit separate responses to the House Budget Committee Health Care Task Force.

### **Improving Medication Management Through Pharmacogenomic (PGx) Testing**

PGx testing is a cornerstone of personalized medicine, providing a way to guide treatment and prevention strategies based on an individual patient's genetic makeup and known drug-gene interactions.<sup>ii</sup> The use of diagnostic tests to detect drug-gene associations can play an important role in avoiding adverse events, optimizing drug dosing, and identifying patients who will or will not respond to certain medications. This information can help guide the application of medicines for many health conditions.

In 2017, the State of Kentucky Teachers' Retirement System partnered with Coriell Life Sciences to provide PGx testing and comprehensive medication management to their patients. Findings published from their experience revealed that the real-world use of PGx testing and active medication management informed by PGx test results leads to more appropriate medication selection.<sup>iii</sup> Not only did patient outcomes improve, but the system saw a shift in health care resource utilization away from acute care services and toward more cost-effective primary care options because the right medicines at the right doses helped keep patients' diseases and conditions in check while helping to avoid adverse drug reactions. For 5,288 patients over 32 months, this led to a reduction of about \$7,000 per patient in direct medical charges and saved the system \$37 million dollars thanks to decreased outpatient and emergency department visits, as well as inpatient hospitalizations.

In mental health, numerous medications approved by the U.S. Food and Drug Administration are impacted by genetics. A growing body of evidence supports the value of PGx-based testing to inform the treatment of mental health disorders. Supporting evidence includes the *PRIME Care* study, which was conducted by the U.S. Department of Veterans Affairs and utilized PGx testing in the treatment of veterans with major depressive disorder (MDD). This study of 1,944 patients who were randomized between treatment guided by PGx testing versus usual care found that after providing PGx testing results to health care providers, patients showed a meaningful decrease in symptoms.<sup>iv</sup> Notably, PGx testing helped veterans with MDD reach symptom remission faster, with patients whose care was guided by PGx testing having higher remission rates during the first 24 weeks compared to those who received usual care.

Clear improvements in patient care and cost savings for health care systems can result from PGx testing and comprehensive medication management strategies. Unfortunately, the number of adverse drug events occurring in this country continues at an alarming rate. The Centers for Disease Control and Prevention report that about 1.3 million emergency department visits and 350,000 hospitalizations in the United States are due to harms from medication use.<sup>v</sup> Many of these situations could be prevented by better integrating genetically informed prescribing and medication management into routine clinical care. In fact, a recent study published in *The Lancet* showed that utilizing PGx testing along with drug-gene interaction guidelines decreased adverse drug reactions by 30 percent.<sup>vi</sup> Adverse drug reactions often lead to avoidable hospitalizations, a meta-analysis of multiple studies showed an average 43 percent reduction in hospitalizations with the use of PGx.<sup>vii</sup> While PGx tests relevant to certain drug-gene associations have been available for more than a decade, routine testing is not widely utilized.

The *Right Drug Dose Now Act of 2023* would direct action across multiple federal agencies to inform the nation's plan to prevent adverse drug events, improve the reporting and collection of PGx information through electronic health records, and facilitate program improvements that would allow regulators to accept this information. To improve patients and providers' awareness and understanding of the potential uses of PGx testing in order to make informed treatment decisions, the *Pharmacogenomics Research and Education Act* also details public awareness and health care professional education campaigns that can help close some knowledge gaps about drug-gene interactions and adverse events. The bill would authorize increased funding for the Genomic Community Resources program to further facilitate the integration of PGx testing into patient care if the full funding amount is appropriated to the National Institutes of Health.

Genetically informed prescribing offers a realistic mechanism to improve individual patient outcomes while reducing overall health care costs. The state of Kentucky recently passed legislation requiring health benefit plans to cover PGx testing for patients. Seven states have passed, and 14 other states are considering similar biomarker screening bills this year. To facilitate meaningful progress toward ensuring that the United States health care system takes full advantage of our increasing understanding of drug-gene interactions at the federal level, **we encourage members of the Health Care Task Force to support reintroduction of the *Right Drug Dose Now Act of 2023* and the *Pharmacogenomics Research and Education Act* by joining as original co-sponsors.**

### **Improving Diagnosis in Rare Disease Through Genetic and Genomic Testing**

Patients with rare undiagnosed diseases, who are often children, currently face a long, invasive, and costly path to diagnosis known as the diagnostic odyssey. Personalized medicine strategies utilizing genetic testing and genomic sequencing can be valuable tools both for discovering changes that contribute to disease development and for accelerating care initiation by health care providers. By reducing the need for additional testing and improving outcomes in patients with treatable rare diseases, economic gains are available to health care systems that embrace the use of genetic and genomic testing to diagnose rare diseases.

A PMC-commissioned study published in April of 2022 suggests that the use of genome sequencing-based tests to profile whole genomes may be the most cost-effective strategy for diagnosing acutely ill infants who are less than a year old with suspected rare genetic diseases.<sup>viii</sup> The study also suggests that under certain assumptions, similar tests may present cost-effective opportunities to uncover disease causes among all children with undiagnosed rare diseases who are under the age of 18. Another economic

study conducted by the EveryLife Foundation for Rare Diseases examined the impact of delayed diagnosis in rare disease and revealed that navigating a rare disease diagnosis can require more than six years and 17 medical interventions after symptoms begin. The diagnostic odyssey costs individuals and families on average over \$220,000 in avoidable medical costs and lost income for individuals and families, and as much as \$517,000 across the seven rare diseases studied.<sup>ix</sup> A preceding study by the EveryLife Foundation for Rare Diseases showed the estimated costs associated with 379 rare diseases reaching nearly \$1 trillion in the U.S. in 2019 between direct health care costs such as inpatient and outpatient care, indirect costs like lost productivity at work and forced retirement, and uncovered health care and non-medical costs.<sup>x</sup> Implementing personalized medicine can help patients with a progressive rare disease receive a faster diagnosis, access better care, and find earlier treatments where available.

Timely diagnosis and screening can shorten and possibly eliminate the diagnostic odyssey while significantly reducing the avoidable costs of rare diseases for individuals, caregivers, and the healthcare system. However, certain barriers, including the lack of insurance coverage, still impede access to genetic testing and genomic sequencing for some patients. **We therefore encourage members of the Health Care Task Force to support reintroduction of the *Precision Medicine Answers for Kids Today Act* by joining as original co-sponsors.**

The *Precision Medicine Answers for Kids Today Act* is intended to address access barriers to diagnostic testing for rare diseases and help the United States maximize the benefits of genetic testing and genomic sequencing for diagnosing diseases among children whose symptoms are suspected to have genetic causes. Through its creation of a three-year demonstration program at the Department of Health and Human Services, the bill would give states an opportunity to cover genetic testing and genomic sequencing and measure how it improves the diagnosis of pediatric health conditions. By requiring the Centers for Medicare and Medicaid Services to report on how often genetic testing and genomic sequencing are covered and reimbursed, the bill aims to fill remaining evidence gaps that currently limit informed decision making. The National Academy of Medicine study detailed in the bill would provide meaningful recommendations for how the federal government can further facilitate patient access to personalized medicine in rare diseases and other disease areas, particularly in marginalized communities.

## Conclusion

PMC appreciates the House Budget Committee Health Care Task Force's interest in examining opportunities to modernize and personalize the health care system. We hope the growing body of clinical and economic evidence supporting broader adoption of personalized medicine and efforts beginning and already underway by Congress to advance this approach to care will be beneficial to more patients and the health system. If you have any questions about the content of this letter, or if PMC can be of further assistance as the Health Care Task Force continues to work in this area, please contact me at 202-499-0986 or [cbens@personalizedmedicinecoalition.org](mailto:cbens@personalizedmedicinecoalition.org).

Sincerely,



Cynthia A. Bens  
Senior Vice President, Public Policy

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- <sup>i</sup> U.S. House of Representatives Committee on the Budget. Letter from Health Care Task Force requesting information on solutions to improve patient outcomes and reduce health spending. August 25, 2023. [https://mcusercontent.com/dcaca8cc3538d2728eecb2399/files/9c3f560b-cb20-0314-65d0-a984f84c6bc8/HBCR\\_Health\\_RFI\\_8.25.2023\\_.pdf](https://mcusercontent.com/dcaca8cc3538d2728eecb2399/files/9c3f560b-cb20-0314-65d0-a984f84c6bc8/HBCR_Health_RFI_8.25.2023_.pdf) (accessed October 10, 2023).
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- <sup>iii</sup> Joseph P. Jarvis et al. “Real-World Impact of a Pharmacogenomics-Enriched Comprehensive Medication Management Program.” *Journal of Personalized Medicine*. March 18, 2022. Vol. 12(3):421. <https://doi.org/10.3390/jpm12030421>.
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- <sup>vii</sup> David V, Fylan B, Bryant E, Smith H, Sagoo GS, Rattray M. An Analysis of Pharmacogenomic-Guided Pathways and Their Effect on Medication Changes and Hospital Admissions: A Systematic Review and Meta-Analysis. *Frontiers in Genetics*. 2021 Jul 30;12:698148. doi: 10.3389/fgene.2021.698148. PMID: 34394187; PMCID: PMC8362615 (accessed October 11, 2023).
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