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Remarks on Public Stakeholder Panel
FDA's Public Workshop on the Seventh Reauthorization of
the Prescription Drug User Fee Act

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Introduction

Thank you to the FDA for the opportunity to share some insights on the importance of the Prescription Drug User Fee program to personalized medicine and to reflect on the *PDUFA VII* goals letter.

I am Cynthia Bens and I serve as Senior Vice President of Public Policy at the Personalized Medicine Coalition, or PMC. PMC is a nonprofit education and advocacy organization that has more than 220 members from across the health care spectrum who are working together to advance personalized medicine in ways that benefit patients with cancer, rare diseases, some common chronic diseases, and infectious diseases.

The *PDUFA* program is a critical source of funding that ensures the timeliness of drug reviews, encourages innovation in drug development, and promotes initiatives at the FDA that leverage the best science. Having a well-resourced, focused, and flexible FDA is essential to achieving PMC's mission of bringing forward the best treatments for each patient and ensuring they are delivered based on that person's biology, medical history, circumstances, and values.

PMC's analyses have shown that personalized medicines account for more than 20 percent of the FDA's new drug approvals each year. That number has steadily increased from 5 percent when the Coalition started looking at approval trends 16 years ago. Initiatives advanced by the FDA in recent years have fostered many notable regulatory firsts including the approval of the first cell and gene therapies, n-of-one therapies, and tissue-agnostic therapies. We believe that enhancements included in the *PDUFA VII* goals letter will advance the future of personalized medicine and that the program will continue yielding benefits for a wide range of patients, including those with unmet medical needs.

It is difficult to summarize all of the reasons why *PDUFA VII* will make meaningful changes for the field of personalized medicine. There are three main areas that I would like to emphasize that PMC is pleased to see included in the *PDUFA VII* goals letter. These areas are targeted resources for staffing to support cell and gene therapy reviews, additional considerations for advancing the use of real-world evidence (RWE) and real-world data (RWD), and the use of digital health tools (DHTs) for regulatory decision-making in support of personalized medicine.

Staffing Needs

In comments provided at the beginning of the *PDUFA VII* process, we highlighted that FDA needed additional resources to develop and maintain a capable and well-trained staff to fulfill the

agency's mission to protect and promote public health while meeting the challenges posed by the increasingly complex regulatory landscape. This need is particularly pronounced in the area of cell and gene-based therapy review.

Cell and gene therapies have the potential to yield unprecedented improvements in clinical outcomes for some disease areas, and they continue to be an important area for personalized medicine. Twenty-two cell and gene therapies have already been approved by the FDA. FDA anticipates that by 2025 it will be reviewing and approving between 10 and 20 cell and gene therapies each year.

PMC has been particularly concerned with the size of the workload facing FDA as a result of the need to evaluate increasing numbers of new cell and gene therapy products. We have called on Congress to provide the FDA with the budget authority appropriations necessary to deal with this workload issue, but realistically meeting FDA's staffing needs for cell and gene therapy work will take funding outside of the appropriations process.

PDUFA VII will allow the addition of significant numbers of FTEs across the FDA divisions by 2027. It is reassuring to us that *PDUFA VII* resources will be devoted to addressing staffing gaps and building capabilities necessary to support the clinical assessment and evaluation of manufacturing processes for cell and gene therapies. PMC appreciates that, in addition to expanding FDA's workforce, attention has been paid in *PDUFA VII* to furthering the development of appropriate regulatory frameworks for assessing the safety and effectiveness of these life-changing therapies.

Real-World Evidence/Real-World Data

We at PMC also believe that data collected about an individual's lifestyle, disease biology, and treatment outcomes can be harnessed to complement traditional clinical trials and can help transform the future of personalized medicine. *PDUFA VI* made some initial improvements at the agency to enhance the use of RWE and RWD, however, important questions remain about how to validate this information and guide its acceptance to support regulatory claims.

In prior comments, PMC supported additional staffing, resources and guidance development under *PDUFA VII* to allow the agency to make further transformations in the use and acceptance of RWE beyond early phase trials and for purposes beyond demonstrating product safety. We are encouraged that the *PDUFA VII* goals letter includes several RWE commitments — such as the proposed pilot program facilitating earlier advice on the quality and acceptability of RWE in support of new labeling claims; a public workshop on RWE; and updates to RWE guidance.

We want the agency to move forward in a science-focused manner, but PMC also recognizes that FDA's thinking on the use of RWE and RWD has been evolving for some time. RWE and RWD can make significant contributions in advancing all of our understanding of which patients will benefit the most from treatments. This trend was not slowed down by the COVID-19 pandemic. Out of necessity, non-traditional approaches to data

gathering in clinical studies were required to facilitate patient participation. Analyses also needed to be conducted on RWD sources to more quickly understand treatment patterns for hospitalized COVID patients.

With the exception of the pilot that begins early in the *PDUFA VII* cycle, the remaining commitments fall near the end. We would urge FDA to accelerate their timeline for RWE and RWD commitments, as it is feasible.

It is promising that participation in the pilot is contingent upon a willingness and agreement with the FDA to publicly disclose elements of an RWE pilot submission. Depending on the level of disclosure, this type of transparency will allow stakeholders to more efficiently leverage real-world datasets. Given the limitations on the size of the pilot and uncertainty about how much information will be disclosed, we also encourage FDA to provide additional avenues for researchers, health data organizations, and other non-industry stakeholders to interact with the agency on RWE and RWD issues.

Digital Health and IT Infrastructure

Finally, PMC called on the FDA at the start of the *PDUFA VII* process to take steps that would accelerate the acceptance of digital health technologies and protocols for decentralized trials. Advances in sensing technologies and self-management platforms have become important tools for personalized medicine. In fact, a growing number of ongoing clinical trials feature the use of wearables and environmental sensors to include more diverse populations, patients in difficult geographic regions, and patients who cannot travel due to the ongoing pandemic.

We applaud the inclusion of several *PDUFA VII* commitments aimed at establishing a framework for promoting regulatory consistency across FDA on DHTs; increasing FDA's capacity in this area; and for hosting a workshop on the use of DHTs for regulatory decision-making leading to DHT guidances. We believe that DHTs can enhance trial efficiency, parallel to the delivery of "real-world" care, and may even provide personalized insights at the point of care.

Much of what we highlight as potential areas for improvement at FDA with respect to RWE, RWD, and DHTs won't be possible without continued modernization of FDA's IT infrastructure. PMC supports the inclusion of *PDUFA VII* resources to increase the agency's capacity to accept and review large datasets as existing needs dictate and as new opportunities emerge.

Conclusion

Thank you again to the FDA for the opportunity to comment on the *PDUFA VII* goals letter on behalf of PMC. My colleagues and I look forward to working with Congress as the *PDUFA* reauthorization process advances to the legislative phase.