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Comments on the Fifth Reauthorization of
the Medical Device User Fee Act (MDUFA V)

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Introduction

Thank you to the U.S. Food and Drug Administration (FDA) for the opportunity to share some insights on the importance of the medical device user fee program to personalized medicine and to reflect on the *MDUFA V* commitment letter.[i]

The Personalized Medicine Coalition (PMC) is a non-profit 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, chronic diseases, and infectious diseases, among others.

The MDUFA program is a critical source of funding. Without MDUFA, many FDA activities that support personalized medicine would not be possible. Having a well-resourced, focused, and flexible FDA is essential to achieving PMC's mission of bringing forward the best treatment and prevention strategies 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 initiatives advanced by the FDA in recent years have fostered many notable regulatory milestones, including in 2021 the Center for Devices and Radiological Health's (CDRH) recognition of the first tumor mutation database allowing test developers to use real-world data to support the clinical validity of new diagnostic tests, as well as the approval of several new diagnostic indications that will allow for targeted treatment decisions for various health conditions.[ii] These new technologies and policies will help innovators and physicians develop safer, more efficacious treatment and prevention regimens based on the principles of patient-centered care.

We believe that enhancements included in the *MDUFA V* commitment letter will help advance the future of personalized medicine and it will continue yielding benefits for a wide range of patients, including those with unmet medical needs. There are three specific areas that PMC is pleased to see in the *MDUFA V* commitment letter. These areas are increased staffing to support CDRH's review activities, additional considerations for advancing the use of real-world evidence (RWE) and real-world data (RWD) and increasing patient engagement and the use of digital health tools.

Staffing Needs

In comments provided at the beginning of the *MDUFA V* process, PMC highlighted that progress made in previous MDUFA reauthorizations have enabled CDRH to reduce the total time it takes to return a decision on a product submission while maintaining high standards for ensuring safety and effectiveness.[iii] This has been possible because of the dedication of CDRH's leadership and staff. It has also been achieved through additional opportunities for engagement between industry and CDRH during the device review process. These interactions allow product sponsors to better understand FDA's data needs. We understand that this level of engagement is only possible if CDRH is properly resourced. PMC supports resource levels in *MDUFA V* that provide adequate staffing to complete the timely evaluation of personalized medicine products that are submitted to CDRH for review.

To help CDRH continue fulfilling its mission to protect public health while meeting the challenges posed by the increasingly complex regulatory landscape, *MDUFA IV* and the *21st Century Cures Act (Cures Act)* included provisions supporting CDRH's efforts to maintain a capable and well-trained staff. FDA has made progress addressing staffing needs, but we understand that challenges remain in attracting and retaining expert staff. In our previous comments on MDUFA's reauthorization, we encouraged CDRH to continue using hiring authorities provided by the *Cures Act* and to pursue enhancements in *MDUFA V* that assist the Center in meeting hiring needs. It is reassuring to us that *MDUFA V* resources will be devoted to current and expanding use of hiring authorities under the *Cures Act*.

Real-World Evidence and Real-World Data

We at PMC believe that data collected about individuals' lifestyles, disease biology and treatment outcomes made available through the collection of real-world evidence (RWE) and real-world data (RWD) can be harnessed to transform the future of personalized medicine. The inclusion of guidance development in *MDUFA IV* on the use of RWE[iv], along with resources to allow CDRH's participation on the Coordinating Committee for the National Evaluation System for health Technologies (NEST) and the Medical Device Innovation Consortium (MDIC), provided a foundation for understanding how and when RWE and RWD may be used to support medical device regulatory decisions. To further FDA's success in this area, PMC called for the inclusion of resources in *MDUFA V* to continue CDRH's involvement in NEST and MDIC. We are pleased to see that the agreement supports these activities, in addition to the issuance of updated guidance providing more clarity on CDRH's RWE activities and at least two public meetings to update stakeholders on the RWE program.

It is also promising that CDRH plans to convene experts to advance innovative methodology approaches with respect to RWE development and analysis and develop best practices. The only stated parties in the commitment letter required to participate in this convening are from industry or appointed by industry groups. Because the impact of CDRH's work in this area extends beyond these entities, we would ask for additional transparency on avenues available for researchers, health data organizations, and other non-industry stakeholders like patient groups to interact with the agency on these two important issues.

Patient Engagement and Digital Health

Identifying the benefits and risks of medical products that matter to patients is essential to the effective delivery of personalized medicine. In 2019, PMC submitted feedback to CDRH on its draft priority list of patient preference-sensitive areas developed as part of *MDUFA IV*. In our comments,[v] we suggested further stakeholder engagement on complicated patient preference-sensitive areas before CDRH proceeds with implementation of a final patient-preference priority list.

PMC generally supports continued work on understanding patients' preferences, and we believe that it can potentially advance activities to positively impact the design and conduct of premarket clinical studies, benefit-risk assessments, and post-market evaluation of medical devices. We appreciate that CDRH's commitments for patient science and engagement under *MDUFA V* include an update to FDA's existing patient preference information guidance that will address common questions for those interested in the voluntary use of patient preference information in regulatory submissions.

PMC also called on the FDA at the start of the *MDUFA V* process to take steps that would accelerate the acceptance of digital health technologies and protocols for decentralized trials. We believe that advances in sensing technologies and self-management platforms are important tools for personalized medicine that should be leveraged in ways that allow for more diverse populations and patients in difficult geographic regions to be included in clinical trials. *MDUFA V* includes commitments to reduce barriers to patient participation and facilitate recruitment and retention in clinical studies by utilizing innovative technologies to capture patient input and reduce patient burden. In addition to supporting this commitment, we look forward to the planned public meeting in FY 2024, which will explore ways to use patient generated health data to help advance remote clinical trial data collection.

Finally, we believe that digital health tools can enhance trial efficiency, parallel to the delivery of real-world care, and may even provide personalized insights at the point of care. We applaud commitments in *MDUFA V* that are intended to ensure that digital health technologies are best considered by fostering the adoption of digital health technologies as evidence generation evolves and to advance understanding of how to measure and monitor software quality. The FDA and external stakeholders will benefit from CDRH's continued work under *MDUFA* V to develop guidances and provide expertise in premarket submissions that include software, interoperable devices, wearables, or digital health technologies like artificial intelligence or machine learning.

Conclusion

Thank you again for the opportunity to comment on the *MDUFA V* commitment letter. My PMC colleagues and I look forward to working with the FDA and Congress as the user fee reauthorization process advances to the legislative phase in the coming months. If there are questions on these comments, please feel free to reach out to me (cbens@personalizedmedicinecoalition.org) or David Davenport, PMC's Manager of Public Policy, (ddavenport@personalizedmedicinecoalition.org).

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[i] U.S. Food and Drug Administration. *MDUFA Performance Goals and Procedures, Fiscal Years 2023 through 2027.March 22, 2022. https://www.fda.gov/media/157074/download*

[ii] Personalized Medicine Coalition. *Personalized Medicine at FDA: The Scope & Significance of Progress in 2021*. February 22, 2022. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/Personalized_Medicine_at_FDA_The_Scope_Significance_of_Progress_in_2021.pdf

[iii] Personalized Medicine Coalition. *Comments on FDA-2020-N-0907-0013 - Medical Device User Fee Amendments (MDUFA) for Fiscal Years 2023 Through 2027.* November 27,2020. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_Comments_MDUFA_V_11.27.20.pdf

[iv] U.S. Food and Drug Administration. *Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices Guidance for Industry and Food and Drug Administration Staff. August 31, 2017.* https://www.fda.gov/regulatory-information/search-fda-guidance-documents/use-real-world-evidence-support-regulatory-decision-making-medical-devices

[v] Personalized Medicine Coalition. *Comments on FDA-2019-N-1619: List of Patient Preference-Sensitive Priorities*. July 12, 2019. http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_Comments_Patient-Preference-Sensitive-Areas-for-Medical-Device-Review.pdf