PERSONALIZED MEDICINE COALITION

November 27, 2020

Jeffrey Shuren, M.D., J.D.
U.S. Food and Drug Administration
Dockets Management Staff (HFA-305)
Fishers Lane, Rm. 1061
Rockville, MD 20852

Sent electronically

Re: FDA-2020-N-0907-0013 - Medical Device User Fee Amendments (MDUFA) for Fiscal Years 2023 Through 2027

Dear Dr. Shuren:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 200 institutions from across the health care spectrum, thanks the U.S. Food and Drug Administration (FDA) for the opportunity to comment on the reauthorization of the *Medical Device User Fee Amendments (MDUFA) for Fiscal Years 2023 through 2027*.[i] PMC appreciates that the *MDUFA* program is an important source of funding for the Center for Devices and Radiological Health (CDRH). Without *MDUFA*, many FDA activities that support personalized medicine would not be possible. PMC's comments are intended to highlight areas for CDRH to consider during the *MDUFA* reauthorization process so that it can continue to accelerate the delivery of personalized medicine.

As you know, 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 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'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.

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Statement of Neutrality

Many of PMC's members will present their own responses to FDA and will actively advocate for those positions. 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 the *MDUFA* reauthorization process.

Staffing CDRH

We understand that *MDUFA* gives FDA the authority to collect user fees from medical product sponsors to support review activities. 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. 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 resources levels in *MDUFA V* that provide adequate staffing to complete the timely evaluation of personalized medicine products that are submitted to CDRH for review. Should CDRH be unable to complete timely evaluations due to a public health emergency, PMC proposes that *MDUFA V* require the FDA to outline a clear, consistent plan with estimated review timelines to improve planning and advance innovation.

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. PMC therefore encourages 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.

We understand that CDRH staff often have limited timeframes to review personalized medicine products for areas of high unmet need, such as companion diagnostics that are paired with an accelerated targeted drug therapy. Despite these constraints, CDRH has innovated to overcome these challenges. However, these learnings have not been shared with the personalized medicine industry to date. Sharing these learnings could allow industry and CDRH to establish best practices that could streamline the process for all parties. To this end, PMC encourages CDRH to consider holding a stakeholder meeting or issuing guidance under *MDUFA V* on various options for industry and reviewers when faced with these challenges.

Advancing Patient Preference Priorities

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 its comments,[ii] PMC suggested further stakeholder engagement on complicated patient preference-sensitive areas like diagnostic testing and direct-to-consumer genetic testing 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. CDRH commitments for patient-preference initiatives under a *MDUFA V* agreement should factor in the need for additional resources to support stakeholder engagement on the impact of incorporating patient-preference information for medical products like diagnostics and genetic testing, where the scientific and regulatory landscapes are rapidly evolving.

Supporting Digital Health Technology

The ubiquity of mobile information devices such as smart phones, advanced sensing technologies and self-management platforms have made them important tools for personalized medicine. A growing number of clinical trials feature the use of wearable and environmental sensors to learn how to deliver real-time care to patients. Digital health platforms such as wearables and mobile apps can help us gather more information and also capture the patient experience, which is a critical perspective. People can report detailed information about their symptoms, treatment burden, quality of life and other experiences, actively and passively documenting their health in detail in ways that go far beyond standard testing performed episodically in a physician's office.

Digital health technologies hold the potential for enhancing trial efficiency, parallel to the delivery of real-world care, and provide personalized insights at the point of care. However, as the adoption of digital health technologies increases, evidence generation may need to evolve. Additionally, better understandings of how to measure and monitor software quality are needed to ensure that digital health technologies are best considered. FDA's recent creation of the Digital Health Center of Excellence (DHCoE) to advance digital health technology innovation and regulation is commendable. We believe CDRH's participation in DHCoE is critical and should be facilitated by *MDUFA V* so that guidance and policy development is coordinated with other FDA centers.

Furthering Real-World Data and Real-World Evidence

Real-world evidence (RWE), or data acquired in everyday clinical practice, can provide valuable insights about an individual's lifestyle, disease biology and treatment outcomes. Thanks to new technologies and data science approaches, this information can be harnessed as a powerful complement to traditional clinical trials. Real-world data (RWD) applications can provide new ways to track disease, allow for optimization of treatment approaches, and capture insights about patient populations to accelerate clinical development. We believe the use of RWE and RWD can help transform the future of personalized medicine if this information can be combined and aggregated in ways that inform answers to questions.

The inclusion of guidance development in *MDUFA IV* on the use of RWE[iii], 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 the foundation for understanding how and when RWE and RWD may be used to support regulatory decisions. To further FDA's success in this area, *MDUFA V* should provide resources for CDRH to continue its involvement in NEST and MDIC. *MDUFA V* should also support the development of standards for the capture and linking of data and the creation of a pilot program to explore the use of RWD obtained from sources in routine care delivery in vitro diagnostic device submissions.

Conclusion

Thank you again for the opportunity to comment on behalf of PMC. My colleagues and I look forward to working with CDRH over the next year through the *MDUFA V* stakeholder consultation process. If you have any questions about the content of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org.

Sincerely yours,

Cynthia A. Bens
Senior Vice President, Public Policy

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[i] U.S. Food and Drug Administration. FDA-2020-N0907-0013: Medical Device User Fee Amendments for Fiscal Years 2023 Through 2027; Public Meeting; Request for Comments. https://beta.regulations.gov/document/FDA-2020-N-0907-0013

[ii] 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

[iii] 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