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Director, Center for Devices and Radiological Health
US Food and Drug Administration
Silver Spring, MD 20993

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Director, Center for Biologics Evaluation and Research
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Re: Comment Letter on Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle [Docket No. FDA-2015-D-1580]

Dear Dr. Tarver and Dr. Marks,

The FasterCures team at the Milken Institute is honored to provide its response to the Request for Comments on Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle [Docket No. FDA-2015-D-1580].

As a nonprofit, nonpartisan think tank, the Milken Institute believes in the power of capital markets to solve urgent social and economic challenges to improve lives. At the heart of the Institute's work is the idea that societies prosper with an educated, healthy workforce, open and efficient capital markets, and effective social institutions. FasterCures is driven by a singular goal: to save lives by speeding scientific advancements to all patients. For the last 15 years, FasterCures has advanced health equity by advocating for systemic patient engagement in biomedical research by bringing together diverse stakeholders to assess gaps, identify solutions, and develop the tools and resources to support decision-making.

FasterCures supports the expanded scope of the draft guidance, which reflects the FDA's expertise in conducting and reviewing patient preference information (PPI) studies for applicable medical devices under the helm of the Center for Devices and Radiological Health (CDRH). The scope is expanded, including the submission and review of investigational device exemption applications, premarket approval applications, 510(k)s, De Novo requests, humanitarian device exemption applications, and various FDA decisions in administrative, enforcement, and other actions. As stated in the title "The Total Product Life Cycle," we believe this reflects the agency's commitment to reinforcing the importance of patient engagement across all aspects of medical device development.

Our recent report, *Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development*, highlights the diversity of the medical technology (medtech) industry, comprising medical devices, diagnostics, and digital health.[1] Such diversity in size, purpose, target population, and users of medtech products renders methodologic uncertainty and unique challenges, which may contribute to growing cultural inertia to pursue patient engagement within the medtech industry.[2] Based on FasterCures' expertise in accelerating biomedical innovation, which is deeply rooted in patient-centricity, we want to share tangible recommendations below to accelerate and improve patient engagement in medtech research and development (R&D).

Summary of Recommendations:

1. Specify the role of patients, caregivers, and patient organizations in the planning and designing of patient preference studies to acquire accurate and meaningful patient preference information.
2. Expand the use of the existing FDA platforms to provide education and resources on patient engagement, particularly for small and medium-sized medtech companies.
3. Empower patients, caregivers, and patient organizations to drive patient engagement in applicable medtech R&D and regulatory processes.

Recommendation 1. Specify the roles of patients, caregivers, and patient organizations in the planning and designing of patient preference studies to acquire accurate and meaningful patient preference information.

In Section V, "Recommendations and Practical Considerations for Patient Preference Studies," we applaud the FDA's recommendation of patient-centeredness by defining that "patient preference studies should ensure that the patient, not the healthcare professional, is the central focus of the study." The agency provided detailed guidance on various factors for the industry to consider in the section. However, FasterCures wants to add one critical factor for the FDA's consideration.

We encourage the agency to go further and recommend that sponsors consider engaging with patients/patient representatives in the early stages of planning and designing patient preference studies. The bar the agency sets for methodological rigor is quite high, but if a patient preference study is not set up to ask the right question(s) in the right way(s) using the right tools/methodologies to reflect patients' actual concerns and engage them effectively, the quality of the output and patient outcomes may not be high or of great value to the FDA. Thus, we recommend that the FDA include an additional bullet in Section IV. I, to read "the timing of patient engagement."

Recommendation 2. Expand the use of the existing FDA platforms to provide education and resources on patient engagement, particularly for small and medium-sized medtech companies.

Patient-Focused Drug Development (PFDD) has gradually solidified as a principle, mandate, and necessity in the R&D of drugs and biologics for the past two decades.[3] Under the 21st Century Cures Act, the FDA is mandated to develop guidance documents regarding the collection and use of patient experience data to inform drug development for applications submitted under sections 505(b) and 351(a) of the Public Health Service Act.

Although not a mandate, medtech sponsors are strongly recommended to consider voluntary patient engagement in clinical studies and the updated guidance document on voluntary patient preference information.[4] The FDA has reinforced its commitment to the science of patient engagement through various User Fee Amendments that continue to engage patients and incorporate their experiences and perspectives in the R&D.

FasterCures' recent report highlights the opportunities and recommendations for stakeholders to drive more effective patient engagement in medtech product development.[5] The medtech industry covers a broad and diverse range of products, and the CDRH has classified roughly 1,700 different generic types of devices.[6] They are grouped by 16 medical specialty "panels"[7] and also classified as Class I, II, and III based on the risk and control needed to ensure the device's safety and effectiveness.[8] Many small and medium-sized companies experience resource constraints, and often patient engagement for their products is not a focus due to competing priorities and uncertainty about the return on investment from engaging patients. Depending on the products, some developers may not have an accessible community of patients or dedicated patient organizations to readily engage as the end-users of their technologies.[9]

FasterCures recommends that the FDA expand the content of its existing education and resource-sharing platforms for small businesses to communicate tailored approaches to patient engagement with clear case studies for the various types of medtech products, including those not traditionally thought of as "patient-facing" products.

Depending on the intended use, levels of risk, and the diversity of users, different depths and types of patient engagement might be appropriate across the total product life cycle. Although the FDA included various case examples in several guidance documents, including the current draft guidance, utilizing the FDA's existing platforms with user-friendly resources will help small and medium-sized medtech companies have a reliable, consistent source for any updates from the agency.

The FDA already has a well-established platform to empower and strengthen small businesses through Regulatory Education for Industry (REdI), which convenes annual conferences for sponsors to share updates and pressing regulatory innovations from the agency's three centers on drugs, biologics, and devices.[10] In addition, each center hosts a small business assistance office. FasterCures sees the platform created by the Division of Industry and Consumer Education at CDRH as a solid platform for the FDA to provide expanded and detailed education and resources regarding patient engagement in the medtech industry.[11] Webinars and educational materials digest the FDA's thinking on patient experience, preference, generated data, and the implication or perhaps incentives for resource-limited sponsors.

Recommendation 3. Empower patients, caregivers, and patient organizations to drive patient engagement in applicable medtech R&D and regulatory processes.

FasterCures recommends that CDRH develop resources and education opportunities for patients, caregivers, and patient organizations to empower them to convey the importance of patient engagement in medtech products, including devices and other health technologies.

The Center has robust patient engagement programs, from listening to patient experiences and perspectives throughout the medical device product life cycle to special advisory committees designated for patient engagement, such as the Patient Engagement Advisory Committee.[12] Its guidance documents provide case examples, Q&A, and design and conduct of meaningful patient engagement throughout medical device clinical studies.[13] We encourage the Center to consider actively educating patient organizations so that they are equipped with guidance and information from CDRH to support proper patient engagement in the medtech industry.

Patients have repeatedly proven that their strong voices can spur change and improvement in the policy and practices of medical products. In the 1980s and 90s, AIDS activists altered the face of medical product development practices and improved the speed of development.[14] More recently, Parent Project Muscular Dystrophy led the development of proposed draft guidance for the industry on rare diseases.[15] Through the 21st Century Cures Act and the FDA's various User Fee Amendments, patient-centric medical product development and scientific and systemic patient engagement have helped create inclusive, scientific, and systemic patient engagement across regulatory processes.[16]

Under the existing platforms, we highly encourage CDRH to build resources directed at increasing the capacity of patients, caregivers, and patient organizations to actively engage with industry sponsors and their health-care providers. The education and resources might include information regarding the patient engagement data in device labeling, where to locate outcomes of clinical trials or studies for medtech products, and patient preference information about the devices under consideration. This increased awareness will allow patients to make more informed decisions when choosing medical products and encourage them to participate in research focused on outcomes that matter to them, thus promoting product development according to patients' needs and preferences.

In summary, FasterCures supports the FDA's expanded scope of its patient preference information guidance based on its years of experience promoting the science of patient engagement in its product evaluation and decision-making. We believe the FDA can strengthen the guidance's focus on the role of patients and patient representatives in generating patient preference information. Additionally, enriching the existing platforms to provide education and resources to the medtech industry and patients will promote the regular and rigorous practice of patient engagement by medical product developers.

Sincerely,

Esther Krofah
Executive Vice President, Health
Milken Institute

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[1] Raymond Puerini, Aneri Suthar, and Kristin Schneeman, *Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development* (Milken Institute, October 2024), https://milkeninstitute.org/content-hub/research-and-reports/reports/defining-and-demonstrating-value-patient-engagement-medtech-research-and-product-development.

[2] Ibid.

[3] "CDER's Patient-Focused Drug Development," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development.

[4] "Patient Engagement for Medical Devices Guidance," U.S. Food and Drug Administration, accessed November 25, 2024. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-engagement-design-and-conduct-medical-device-clinical-studies; "Revisions to 2016 PPI Guidance," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle.

[5] Puerini et al., *Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development*.

[6] "How to Determine If Your Product Is a Medical Device," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/medical-devices/classify-your-medical-device/how-determine-if-your-product-medical-device.

[7] "Device Classification Panels," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/medical-devices/classify-your-medical-device/device-classification-panels.

[8] "Classify Your Medical Device," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/medical-devices/overview-device-regulation/classify-your-medical-device.

[9] Puerini et al., *Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development*.

[10] "REdI Conference," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/medical-devices/contact-us-division-industry-and-consumer-education-dice/redi-conference.

[11] "Device Advice: Comprehensive Regulatory Assistance," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/medical-devices/device-advice-comprehensive-regulatory-assistance.

[12] "CDRH Patient Engagement," U.S. Food and Drug Administration, accessed November 25, 2024. https://www.fda.gov/about-fda/division-patient-centered-development/cdrh-patient-engagement.

[13] "Patient Engagement for Medical Devices Guidance," U.S. Food and Drug Administration; "Patient-Reported Outcome Measures: Use in Medical Product Development," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims; "Revisions to 2016 PPI Guidance," U.S. Food and Drug Administration, accessed November 25, 2024, https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle.

[14] Nurith Aizenman, "How to Demand a Medical Breakthrough: Lessons from the AIDS Fight," NPR, February 9, 2019, https://www.npr.org/sections/health-shots/2019/02/09/689924838/how-to-demand-a-medical-breakthrough-lessons-from-the-aids-fight.

[15] Pat Furlong, et al., "How a Patient Advocacy Group Developed the First Proposed Draft Guidance Document for Industry for Submission to the U.S. Food and Drug Administration," *Orphanet Journal of Rare Diseases* 10, no. 82 (June 2015), https://ojrd.biomedcentral.com/articles/10.1186/s13023-015-0281-2.

[16] "Public Law 114–255 114th Congress," congress.gov, accessed November 25, 2024, https://www.congress.gov/114/plaws/publ255/PLAW-114publ255.pdf.