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 via electronic delivery

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

Dear Dr. Shuren:

On behalf of the patients impacted by rare diseases, the EveryLife Foundation is pleased to offer the following comments regarding the Medical Device User Fee Amendments (MDUFA) for Fiscal Years 2023 through 2027. The EveryLife Foundation for Rare Diseases is a nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.

The inherent nature of rare diseases can make them difficult to diagnose, with small patient populations and limited investment in medical research and device development. Efforts to increase research and development are ongoing, but according to an Institute of Medicine Report, *Rare Diseases and Orphan Products: Accelerating Research and Development*[i], those efforts "clearly focus on drugs and biological products. Devices and the need for devices are much less frequently mentioned in journal articles and stakeholder conversations." A joint FDA/NIH report, *Unmet Medical Device Needs for Patient with Rare Diseases*[ii], states that "device development for rare diseases significantly lags behind orphan drug development." In that same report, 77 percent of clinicians cited a need for entirely new diagnostic and/or therapeutic devices, with 64 percent dissatisfied with existing diagnostic and/or therapeutic devices. There is a need to continue to grow the number of medical devices available to the rare disease community to improve both therapeutic and diagnostic options for the entire community.

As we plan for MDUFA V, we have identified three priority areas for further consideration:

- Building upon patient engagement efforts and improving the use of patient experience data in decision making

- Improved incentives for rare disease device development through the Humanitarian Use Device Program
- Maximizing digital health opportunities for the diagnosis and treatment of rare disease

Building On Existing Patient Engagement Efforts and Improving the Integration of Patient Experience Data in Decision Making

CDRH has long been a leader in patient engagement efforts and the commitments made during MDUFA IV built upon that legacy. CDRH has been a leader in committing to robust patient engagement through the Patient Engagement Advisory Committee and the Patient and Caregiver Connection Programs. In addition, the advancement in the conduct and use of patient preference information is directly attributable to the Center's commitment to meaningful incorporation of patient experiences in the regulatory process.

Even with this great progress, there are areas CDRH can continue to improve upon related to patient engagement and the use of patient preference studies in people with rare disease. While we recognize the added difficulty of maintaining a commitment to patient engagement activities during the COVID-19 pandemic, we urge you to work with the patient communities to identify new ways to convene, additional methods to ensure that diverse and inclusive populations are heard, and the safety of participants is protected, even after the public health emergency concludes, as vulnerable populations will likely be isolated for extended periods of time.

We urge CDRH to consider the following as you plan your patient engagement initiatives:

- Aligned with comments offered by EveryLife at the August 2020 PDUFA VII Public Stakeholder Workshop, we encourage CDRH to help ensure the robust data gathered through your patient engagement activities and the use of patient preference studies in regulatory submissions is optimized for maximum benefit of the community. If made available in the right formats, this information can inform outcome measure development, identify unmet needs, and encourage further investment by developers and patient organizations while also informing patient centered value and access decisions following approval. We urge you to consider instituting a standard reporting mechanism for how patient experience and preference data is informing the review of devices that are approved.

- In addition, we encourage CDRH to expand their work in patient preference studies for novel rare diseases. Patient preference studies are vital for ensuring that patient voice is heard during the device development process and should be encouraged even for the small populations associated with rare disease device development. We hope to collaborate with the Office of Orphan Drug Products to encourage and support the conduct of patient preference studies for novel rare disease devices.

- Lastly, we encourage you to further efforts to engage review advisory committee members on CDRH's commitment to patient engagement and patient preference studies, including identifying the most effective ways to present this information to advisory committee members as part of their review. Advisory Committee members provide a tremendous service to the public with their rigorous, expert reviews. Their reviews play an important role in the incorporation of patient information, but since the science of patient input is relatively new, committee members may not yet have the depth of expertise to fully understand opportunities to integrate patient experience data when informing decision making. If not presented clearly and with the appropriate instructions for how to use the information, there could be unintended consequences that negatively impact the Advisory Committee's discussions. Streamlining how patient preference information is presented and providing some focused content area training would be an impactful way to encourage additional developers to include such information in their submissions.

Improved Incentives for Rare Disease Device Development Through the Humanitarian Use Device Program

We would also like to underscore the significant unmet need within the rare disease community related to the development of novel devices. The Humanitarian Use Device (HUD) program has long aspired to help to bring much needed devices to the rare disease community. The expansion of the exemption to include 8,000 individuals was a welcomed change, bringing the program closer in line with the orphan drug standard of affecting less than 200,000 patients in the United States, but its utilization falls far short of the need.

There is great promise in device/drug combinations for rare disease treatment. Earlier engagement and clarification on which combination products are eligible for HUD and the interpretation of the probable benefit standard will help to bring much needed treatment to the community.

We urge you to consider HUD reforms during the MDUFA negotiations and we look forward to working with the Office of Orphan Products Development and CDRH to find ways to continue to grow the impact of the HUD program so that more device companies are incentivized to develop products for rare disease indications.

Maximizing Digital Health Opportunities for the Diagnosis and Treatment of Rare Disease

Finally, we want to thank you for all the great work that is occurring at CDRH around innovations in digital health. We are pleased by the launch of the Digital Health Center of Excellence and eager to see all the great work the Center will do in setting international standards on the issue.

As the Center leadership build on this work, we urge you to keep the needs, experiences, and preferences of those with rare diseases a key priority. Artificial intelligence and machine learning hold great promise for shortening the diagnostic odyssey for so many with rare diseases. Additionally, CDRH should consider how these AI tools can be used to accelerate therapy development for rare diseases. Other digital technology and devices have the potential to connect people with rare diseases to care and clinical trials that were otherwise unattainable. We hope that you will engage with the rare disease community, including patients and caregivers who would use new digital health tools and the specialists treating people with rare diseases as you build out appropriate regulatory frameworks so that you can identify unique perspectives and unique flexibilities that might be required for these populations.

In closing, we are grateful to the FDA for your leadership and continued commitment to placing patients at the heart of product development – and we look forward to continuing to collaborate and innovate alongside you to ensure the needs of the rare disease community are met going forward.

The EveryLife Foundation for Rare Diseases would be happy to serve as a resource or to facilitate engagement with rare disease community partners; please contact Jamie Sullivan, Director of Policy at jsullivan@everylifefoundation.org.

Sincerely,

Julia Jenkins
Executive Director
EveryLife Foundation for Rare Diseases

Annie Kennedy
Chief of Policy & Advocacy
EveryLife Foundation for Rare Diseases

CC:
Mark Dant, Board Chairman
Frank J. Sasinowski, Board Vice-Chairman

[i] Institute of Medicine. 2010. Rare Diseases and Orphan Products: Accelerating Research and Development. Washington, DC: The National Academies Press. https://doi.org/10.17226/12953.

[ii] Food and Drug Administration and National Center for Advancing Translational Science, 2018. *Unmet Medical Device Needs for Patients with Rare Diseases.* https://www.fda.gov/media/111309/download

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