June 30, 2026

SUBMITTED ELECTRONICALLY

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: Muscular Dystrophy Association Comments on "Impacts of Patient-Focused Drug Development Meetings; Establishment of a Public Docket; Request for Information and Comments" - Docket No. FDA-2026-N-3947

To Whom It May Concern;

In service of the neuromuscular disease (NMD) community, the Muscular Dystrophy Association (MDA) thanks the Food and Drug Administration (FDA or "Agency") for the opportunity to comment on the Agency's "Impacts of Patient-Focused Drug Development Meetings; Establishment of a Public Docket; Request for Information and Comments". MDA has supported the Patient-Focused Drug Development (PFDD) initiative since its inception, has hosted one externally-led PFDD meeting focused on Pompe disease in July 2020, and has participated in several neuromuscular disease PFDD meetings hosted by collaborators. Consequently, we are pleased to offer our experiences and perspectives to the Agency.

MDA is the #1 voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For over 75 years, MDA has led the way in accelerating research, advancing care, and advocating for the support of our community. MDA's mission is to empower the people we serve to live longer, more independent lives.

The Patient-Focused Drug Development initiative, including FDA-hosted and externally-led PFDD meetings, is the one of the most salient and impactful ways for the neuromuscular disease community to inform FDA decisionmakers on their benefit/risk considerations, their willingness to participate in clinical research, and the meaningfulness of clinical endpoints, outcome assessments, and trial designs. The Agency should be well aware of the importance of the PFDD initiative to not only the NMD community, but the rare and chronic disease communities broadly speaking. As we answer the questions posed by the Agency in this "Request for Information and Comments", MDA's strong support for the initiative and its continuance should be well understood.

1. **How have PFDD meetings informed patient communities and stakeholder engagement activities?**

PFDD meetings have been valuable in informing patient communities and stakeholder engagement activities in various ways. During our Pompe disease PFDD meeting, stakeholders were able to learn from each other, including in the various ways in which they have adapted their lives to living with the disease, and learning from each other's clinical and research decisionmaking approaches.

Pompe is also an evolving disease due to the presence of several FDA-approved treatments as well as near-universal newborn screening. In the not-so-distant future, no adult should be diagnosed with late-onset Pompe disease. Instead, anyone newly diagnosed with Pompe should be diagnosed shortly after birth, thus dramatically changing the experience of living with the disease. This was all covered during the Pompe PFDD meeting as it relates to daily living experiences and clinical and research decisionmaking.

Similar outcomes have arisen from PFDD meetings for myotonic dystrophy (hosted by the Myotonic Dystrophy Foundation), facioscapulohumeral dystrophy (FSHD, hosted by the FSHD Society), spinal muscular atrophy (hosted by CureSMA), and Friedreich's Ataxia (hosted by FARA), just to name several meetings that occurred within the neuromuscular disease field. These meetings also illustrated who within each community may be experiencing their disease differently than the majority (or least most vocal majority) of the community, and consequently how their benefit/risk considerations and other viewpoints on living with their disease may differ.

2. **What scientific questions, research initiatives, or identified gaps in the understanding of a disease or condition have resulted from PFDD meetings?**

Various questions, initiatives, and gaps in the understanding of a disease have resulted from PFDD meetings, including the Pompe disease PFDD meeting. We are confident that following our meeting, attendees and the drug development and regulatory communities had a better understanding of who may be willing to participate in clinical studies and when. Parents of children with newly-diagnosed LOPD, newly found through newborn screening, and how they are approaching their child's disease were of particular interest.

The Pompe community also successfully emphasized the importance of respiratory function as an endpoint in clinical trials, hopefully pushing the field towards respiratory endpoints. The timeline of optimal treatment administration was discussed in the context of FDA regulatory approvals, a conversation that may have contributed understanding to a previously under-discussed topic.

3. **In what ways has patient input from PFDD meetings informed medical product development programs or strategies (e.g., endpoint development, clinical trial design, identification of unmet needs, industry partnerships)?**

Admittedly it can be difficult to ascertain when the takeaways from a PFDD meeting, including the findings published in a Voice of the Patient Report, have a direct impact on medical product development programs and strategy. Companies developing treatments consider a myriad of factors as they are choosing endpoint development, clinical trial design, unmet needs, and partnerships, and their usual public communications state exactly that.

Additionally, while the data collected and presented within Voice of the Patient Reports are valuable, in a rapidly evolving rare disease ecosystem, the data may become out of date relatively quickly. Perhaps new therapies have been approved or the promise of the pipeline has influenced the community's risk/benefit calculations. This may make it difficult for sponsors to confidently use patient input from PFDD meetings several years after their occurrence.

5. **Please describe any other outcomes or effects resulting from PFDD meetings, with examples, that were not addressed in the questions above.**

PFDD meetings have informed who within the community may feel left out of existing initiatives. For example, during the FSHD PFDD meeting hosted by the FSHD Society, younger individuals diagnosed earlier and with faster-progressing symptoms expressed that existing programs may not be serving them and their experiences.

PFDD meetings have also informed our community on what information gaps may remain in the FDA or other key decisionmaker's understanding of the disease. For example, following the FSHD PFDD meeting, the FSHD Society also held a listening session focused on upper-limb mobility. Other communities followed their PFDD meeting with information collection and outreach focused on gene therapies. Others still may have focused their efforts on subpopulations that may not have gotten as much time dedicated to their experience in the PFDD meeting.

All of this points to the importance of the FDA engagement opportunities following a PFDD meeting (including patient listening sessions, scientific meetings, informational meetings held with specific review divisions, and other mechanisms), and how helpful it would be for the Agency to offer greater guidance on how each of these opportunities (and more) can be utilized following a PFDD meeting.

Greater clarity would also be helpful in how organizations who have hosted PFDDs and their communities can update the information presented in the PFDD meeting if it at any point goes out of date. We want the FDA and the biopharmaceutical stakeholder community to have the most up-to-date information and viewpoints available, but after a Voice of the Patient Report is published, it is unclear on how we can update the information contained within.

We are grateful for the opportunity to comment on the FDA's request for comment or information on Patient-Focused Drug Development meetings. For questions regarding MDA or the above comments, please contact me at pmelmeyer@mdausa.org

Sincerely,

Paul Melmeyer, MPP
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Muscular Dystrophy Association