MYOSITIS SUPPORT & UNDERSTANDING
EMPOWERING THE MYOSITIS COMMUNITY

Re: FDA-2026-N-3947 — Public Comment on Externally-Led Patient-Focused Drug Development (EL-PFDD) Meetings

Submitted by:
Myositis Support and Understanding (MSU)

Introduction

Myositis Support and Understanding (MSU) is a patient-led organization dedicated to improving the lives of individuals affected by idiopathic inflammatory myopathies (myositis), including dermatomyositis (DM), polymyositis (PM), inclusion body myositis (IBM), and related conditions.

In June 2024, MSU, in collaboration with The Myositis Association (TMA), convened an Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting on Adult Dermatomyositis. This meeting brought together patients, caregivers, clinicians, and FDA representatives to capture the lived experience of individuals with DM and to identify key unmet needs in treatment, clinical care, and research.

We appreciate the opportunity to provide comments to the FDA on the impact of EL-PFDD meetings and to share our perspective on how these efforts can continue to evolve.

Impact of EL-PFDD Beyond the Meeting

The Adult Dermatomyositis EL-PFDD meeting has had a meaningful impact beyond the initial public meeting, demonstrating that externally led PFDD efforts can extend into ongoing research, clinical development, and patient advocacy activities.

Following the meeting, MSU developed a comprehensive Voice of the Patient (VoP) Report, supported by a pre-meeting survey (n=198), structured polling, and patient and caregiver testimonies. These data have been further organized into analyzable datasets and are currently being used to support scientific dissemination, including manuscript development and secondary analyses.

The EL-PFDD process also catalyzed internal capacity-building within MSU, including the development of structured data dictionaries, analytical frameworks, and plans for integration into a broader patient registry initiative. These efforts reflect a transition from a one-time engagement to a sustained patient-centered evidence generation platform.

Role of the Voice of the Patient Report as a Living Scientific and Advocacy Resource

The Adult Dermatomyositis EL-PFDD meeting has demonstrated that Voice of the Patient (VoP) Reports can serve not only as summaries of patient input at a single point in time, but as *living scientific and advocacy resources* that continue to inform stakeholders well beyond the initial meeting.

Following the June 2024 EL-PFDD meeting, the resulting VoP Report and associated patient-generated datasets have been actively utilized by multiple stakeholders, including patient organizations, academic investigators, and medical product developers. These stakeholders have engaged with the findings to better understand patient priorities, symptom burden, treatment experiences, and unmet needs in dermatomyositis.

Importantly, the VoP Report has supported ongoing dialogue between patient organizations and sponsors developing novel therapies—including advanced modalities such as cell-based therapies and targeted immunotherapies—regarding patient-centered endpoints, benefit-risk considerations, and clinical trial design. **These data have also informed internal evidence generation strategies among sponsors**, reflecting the practical value of EL-PFDD outputs in shaping research and development activities.

In several instances, sponsors have expressed interest in conducting secondary analyses of the underlying patient datasets to further explore these domains in a structured and quantitative manner.

This evolving use of the VoP Report illustrates that EL-PFDD outputs can function as dynamic, reusable evidence sources that contribute to research planning, evidence generation, and patient-centered development strategies. Rather than remaining static documents, VoP Reports can serve as foundational assets that support longitudinal learning and continuous engagement with the patient community.

From an advocacy perspective, the VoP Report also provides a credible, FDA-recognized platform through which patient voices can be consistently represented in scientific and regulatory discussions. This enables patient organizations to move beyond anecdotal input and contribute structured, data-driven insights that are increasingly being considered in clinical development and decision-making processes.

Evidence of Influence on Drug Development and Clinical Research

MSU has observed that the Adult Dermatomyositis EL-PFDD outputs are increasingly being incorporated into the broader therapeutic development landscape.

Sponsors developing therapies for dermatomyositis—including biologics, targeted immunotherapies, and emerging cell-based therapies—have engaged with MSU to better understand findings related to:

- Symptom burden, including fatigue, muscle weakness, and skin disease
- Treatment experiences, including incomplete efficacy and treatment-related side effects
- Patient priorities for future therapies, including preferences for administration and disease-modifying potential

- Clinical trial participation barriers, including travel burden, eligibility constraints, and placebo concerns

These interactions have extended beyond general awareness to more structured discussions, including exploration of secondary analyses of patient-reported datasets and consideration of how these findings may inform clinical trial design and endpoint selection.

In parallel, the EL-PFDD datasets have supported ongoing research efforts, including the preparation of peer-reviewed publications and development of structured analytical frameworks that enable more rigorous interpretation of patient experience data.

Recommendations for the FDA

Based on our experience, we respectfully offer the following recommendations to further enhance the impact of EL-PFDD efforts:

1. Recognize EL-PFDD outputs as durable evidence resources
FDA should acknowledge that VoP Reports and associated datasets can serve as ongoing sources of patient experience evidence, rather than static meeting summaries.

2. Encourage sponsors to incorporate EL-PFDD findings early in development
FDA guidance and interactions (e.g., pre-IND, end-of-Phase 2 meetings) should encourage sponsors to reference EL-PFDD findings when designing trials and selecting endpoints.

3. Support secondary analysis of EL-PFDD datasets
FDA should recognize and encourage collaborations between patient organizations and sponsors to perform secondary analyses of EL-PFDD data, enabling deeper and more quantitative insights. Including data surveys conducted concomitantly with the event.

4. Promote integration with patient registries
FDA should support efforts to extend EL-PFDD findings through longitudinal data collection, including patient registries that can capture disease progression, treatment experience, and outcomes over time.

5. Recognize patient organizations as data stewards
Patient organizations play a critical role not only in convening EL-PFDD meetings but also in curating, maintaining, and interpreting patient experience data. FDA should recognize this role in future guidance and engagement model

Conclusion

The Adult Dermatomyositis EL-PFDD meeting demonstrates that externally-led PFDD efforts can evolve into sustained, patient-centered contributions to the scientific and regulatory ecosystem.

The Voice of the Patient Report and associated datasets have become living resources that continue to inform stakeholders across research, clinical development, and advocacy. These efforts highlight the importance of viewing patient experience data not as a one-time input, but as an ongoing foundation for understanding disease burden, shaping therapeutic innovation, and improving outcomes for patients.

We appreciate the FDA's continued leadership in advancing patient-focused drug development and welcome further opportunities to contribute to this important work.

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