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June 30, 2026

U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

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

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to submit comments regarding the request for information and comments on the Agency's **Impacts of Patient-Focused Drug Development Meetings; Establishment of a Public Docket**.

BIO is the premier biotechnology advocacy organization representing biotech companies, industry leaders, and state biotech associations in the United States and more than 35 countries around the globe. BIO members range from biotech start-ups to some of the world's largest biopharmaceutical companies – all united by the same goal: to develop medical and scientific breakthroughs that prevent and fight disease, restore health, and improve patients' lives. BIO also organizes the BIO International Convention and a series of annual conferences that drive partnerships, investment, and progress within the sector. Learn more at bio.org.

Sincerely,

/s/
Neil Ichiro Laruan
Director, Science & Regulatory
Biotechnology Innovation Organization

General Comment(s):

BIO appreciates the opportunity to share industry experience regarding how Patient-Focused Drug Development (PFDD) meetings have informed activities beyond individual regulatory decisions. These meetings have become an important component of the broader patient engagement landscape by providing opportunities for patients and caregivers to describe the realities of living with their conditions in a setting that brings together regulators, patient organizations, researchers, and medical product developers. The resulting discussions often provide insights not readily captured in the traditional scientific literature, including the day-to-day burdens of disease, treatment experiences, and the outcomes patients consider most meaningful.

BIO believes the value of PFDD meetings extends well beyond the meetings themselves. Voice of the Patient reports and other meeting outputs have served as enduring resources that inform interactions with patient organizations, support evidence-generation strategies, and provide context regarding unmet need across therapeutic areas. In many cases, these resources provide one of the few publicly available summaries of patient priorities developed through a structured engagement process, allowing stakeholders to establish a common understanding of disease burden and treatment goals. Particularly in disease areas where opportunities for large-scale patient engagement may be limited, these resources can provide an important foundation for understanding patient priorities and informing future development efforts.

BIO recognizes that PFDD outputs provide insight into patient perspectives at a particular point in time. As standards of care evolve and new therapeutic options emerge, patient priorities and treatment expectations may also change. For this reason, Voice of the Patient reports are often viewed as important reference points that may benefit from periodic reassessment and supplementation through additional patient engagement activities. Maintaining the relevance of these resources will help ensure they continue to reflect contemporary patient experiences and remain useful in informing future research and development activities.
BIO also encourages FDA to enhance transparency regarding how patient experience data are considered throughout the regulatory process. Greater clarity regarding how these data inform review discussions, why submitted evidence may be considered insufficient for a particular regulatory purpose, and how patient experience data contribute to benefit-risk assessments would strengthen future evidence-generation efforts and support continued investment in high-quality patient experience data. Where appropriate, FDA should consider leveraging existing review documents, such as Benefit-Risk Frameworks and Summary Basis of Approval documents, to more explicitly describe how patient experience data were evaluated and incorporated into regulatory decision-making. Such transparency would help sponsors and patient organizations better align future evidence-generation efforts with regulatory expectations.

Specific Questions:

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

BIO has found that PFDD meetings create opportunities for engagement that might not otherwise occur through conventional scientific or regulatory forums. By bringing together patients, advocacy organizations, sponsors, healthcare providers, and regulators around a common set of discussion topics, these meetings help establish a shared understanding of disease burden, unmet need, and treatment priorities. Participation in PFDD activities has frequently served as the foundation for longer-term relationships with patient organizations and encouraged continued dialogue throughout the development process. These meetings have also provided important insight into disease areas, particularly rare diseases, where published literature and opportunities for broad patient engagement may be limited, helping establish a stronger understanding of patient experiences early in product development.

PFDD meetings have strengthened collaboration among industry, patient advocacy organizations, and regulators by creating opportunities for early input into discussion topics and priorities. Through participation in externally led PFDD meetings, stakeholders have worked together to ensure that issues most relevant to patients are reflected in broader discussions regarding disease burden, treatment experiences, and unmet need. These interactions have fostered ongoing engagement among patient communities, advocacy organizations, researchers, and medical product developers and have informed subsequent educational and evidence-generation activities.

PFDD meetings have further expanded understanding of patient experiences that may not be readily apparent through traditional engagement activities. In multiple sclerosis, for example, discussions highlighted challenges associated with accessing specialized care, with some patients describing what they characterized as a "neurology desert." By capturing experiences such as these, PFDD meetings help raise awareness of the barriers patients face and provide a forum for stakeholders to understand better how disease impacts individuals beyond clinical settings.

The publicly available nature of Voice of the Patient reports has further amplified the impact of these meetings. These reports continue to inform educational initiatives, advocacy efforts, and discussions regarding unmet need long after the meetings conclude. By establishing a shared understanding of patient priorities, they provide a common reference point for patients, sponsors, researchers, and regulators. For rare diseases and conditions with limited published literature, PFDD meetings may offer one of the few structured opportunities to capture and disseminate patient perspectives in an accessible manner to the broader community, helping to increase awareness of diseases that may otherwise receive limited attention.

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

BIO has found that patient perspectives shared during PFDD meetings often identify scientific questions and knowledge gaps that may not emerge through traditional clinical research approaches alone. By providing insight into lived experiences, these discussions have highlighted aspects of disease burden, symptom variability, treatment experiences, and functional impacts that warrant additional investigation. Across therapeutic areas, the information generated through these meetings has helped identify gaps in understanding disease progression, caregiver experiences, and the outcomes that matter most to patients.

One recurring theme has been the need to better understand whether existing clinical measures adequately capture the heterogeneity of patient experiences. Discussions across disease areas have highlighted situations in which traditional assessments may not fully reflect the day-to-day impact of disease on patients' lives. For example, discussions in multiple sclerosis highlighted a disconnect between standard clinical measures. They lived experience, with patients describing ongoing fatigue, cognitive challenges, pain, and functional limitations despite appearing clinically stable according to imaging findings or physician assessments. These experiences reinforced the importance of developing approaches capable of more comprehensively evaluating disease burden, disease progression, and treatment experiences most meaningful to patients.

Patient perspectives have also highlighted symptoms and impacts that may be underrecognized despite their substantial effect on quality of life. In multiple sclerosis, discussions identified challenges associated with heat sensitivity, cumulative mental health burden, and uncertainty regarding disease progression. More broadly, these conversations demonstrated how PFDD meetings can reveal dimensions of disease burden that may not be routinely captured through traditional research approaches and can help inform future evidence-generation priorities.

Additional insights have emerged in rare diseases, where published literature and clinical experience may be more limited. For example, discussions in hypophosphatasia contributed to a broader understanding of disease manifestations and caregiver burden, helping contextualize unmet need and identify opportunities for future research. These perspectives reinforced the value of natural history studies and highlighted the importance of generating evidence that reflects both patient and caregiver experiences.

Taken together, these discussions have reinforced the importance of developing fit-for-purpose clinical outcome assessments that accurately reflect concepts of importance to patients. The scientific questions identified through PFDD meetings have not only deepened understanding of disease burden but have also informed future research priorities and highlighted opportunities to improve the measurement of outcomes meaningful to patients.

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)?**

Industry experience has demonstrated numerous ways in which patient input from PFDD meetings has informed medical product development programs and strategic decision-making. In some instances, the impact has been direct, influencing endpoint selection or evidence-generation plans. In others, PFDD outputs have served as foundational resources that provide context regarding disease burden, unmet need, and the outcomes patients consider most meaningful. Collectively, PFDD meetings have fostered a more patient-centered approach to medical product development by encouraging earlier and more systematic integration of patient perspectives throughout the product lifecycle.

Patient perspectives shared during PFDD meetings have informed discussions regarding endpoint development and the selection of concepts of interest that underpin clinical outcome assessment strategies. Narratives regarding symptom burden, functional limitations, and treatment experiences have helped identify outcomes that are meaningful to patients and therefore warrant consideration within development programs. In some cases, Voice of the Patient reports have been referenced during regulatory interactions to provide context regarding unmet need and support the rationale for proposed measurement approaches. More broadly, these discussions have reinforced the importance of ensuring that endpoints and outcome measures reflect concepts of interest that are meaningful and relevant to patients.

Insights generated through PFDD meetings have also informed broader development strategies and evidence-generation activities. Across therapeutic areas, discussions have highlighted the importance of understanding treatment burden, patient preferences, caregiver perspectives, and varying levels of risk tolerance when designing development programs. For example, patient perspectives shared in conditions such as amyotrophic lateral sclerosis (ALS), complement 3 glomerulopathy (C3G), and xerostomia have provided important context regarding symptom burden, treatment experiences, and unmet need that can inform endpoint selection, benefit-risk assessments, and future development planning. These discussions have also highlighted opportunities to better understand treatment sequencing, long-term disease management, and outcomes that may not be adequately captured through traditional clinical measures.

PFDD meetings have also encouraged earlier and more continuous engagement with patients and patient organizations throughout medical product development. Rather than viewing patient engagement as a discrete activity associated primarily with regulatory interactions, many organizations increasingly seek patient input during early development planning, protocol development, clinical outcome assessment development, and study feasibility evaluations. This evolution reflects a broader shift toward integrating patient experience throughout development and has strengthened the scientific rationale and patient relevance of development programs.

Patient input has further informed clinical trial design by identifying opportunities to reduce participant burden and improve the overall trial experience. Discussions regarding treatment burden, visit frequency, travel requirements, caregiver responsibilities, and the impact of disease on daily activities have informed efforts to simplify study procedures, strengthen

recruitment and retention strategies, and ensure that development programs better reflect the realities patients and caregivers face.

PFDD meetings have additionally strengthened collaborations among sponsors, patient advocacy organizations, academic researchers, clinicians, and other stakeholders. These interactions have helped establish partnerships that support natural history studies, patient experience data collection, development of clinical outcome assessments, and other research initiatives that may benefit future development programs. By fostering greater collaboration across the medical product ecosystem, PFDD meetings have contributed to a more comprehensive understanding of patient needs and encouraged more coordinated approaches to therapeutic innovation. These experiences demonstrate that PFDD meetings can shape not only specific development decisions but also broader organizational approaches to integrating the patient voice throughout the medical product lifecycle.

4. **How has patient input gathered during PFDD meetings been integrated into or otherwise affected clinical practice?**

While PFDD meetings are not primarily designed to influence clinical practice, the insights generated through these discussions frequently highlight opportunities to improve patient-provider communication and promote more patient-centered care. By elevating aspects of disease burden that may not be routinely discussed during clinical encounters, PFDD meetings can contribute to a more comprehensive understanding of the patient experience and help identify areas where additional dialogue, education, or support may be beneficial.

One recurring theme emerging from PFDD discussions is the potential difference between how patients and healthcare providers perceive disease management and treatment success. Patients have described adapting to symptoms and functional limitations that may not always be apparent through traditional clinical assessments, even when their condition is considered well-managed from a clinical perspective. These conversations underscore the importance of understanding how patients define treatment success and highlight opportunities to incorporate patient perspectives into shared decision-making discussions more intentionally.

PFDD meetings have also created opportunities for patients to discuss symptoms and concerns that may be underrecognized despite their substantial impact on quality of life. For example, discussions in multiple sclerosis highlighted challenges related to sexual health, bladder dysfunction, and incontinence, illustrating how patient perspectives can broaden understanding of disease burden beyond traditional clinical measures. In rare diseases and conditions with rapidly evolving treatment landscapes, these discussions have similarly highlighted opportunities to strengthen awareness of available therapies, emerging evidence, and patient support needs among both patients and healthcare providers.

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

BIO identifies several broader impacts of PFDD meetings that extend beyond specific research initiatives or individual development programs. One of the most significant outcomes has been the creation of publicly available resources that provide a shared foundation for understanding patient priorities and unmet needs. Voice of the Patient reports have become important reference documents across the patient engagement and medical product development ecosystem, informing ongoing discussions about disease burden, treatment expectations, and patient experiences. By establishing a common understanding of patient priorities, these resources allow subsequent discussions to focus more directly on how unmet needs may be addressed through research, development, and regulatory decision-making.

PFDD outputs have also been referenced beyond regulatory and product development activities. In some cases, patient experience information generated through externally led PFDD meetings has been used to inform coverage and reimbursement discussions by providing structured evidence on disease burden, treatment experiences, and patient-important outcomes. For example, patient perspectives generated through EL-PFDD meetings have been cited in coverage determinations for conditions such as sickle cell disease and Duchenne muscular dystrophy, illustrating how these resources may help inform access-related decision-making across the healthcare ecosystem.

The influence of PFDD outputs increasingly extends beyond the United States. Although developed within the context of FDA initiatives, Voice of the Patient reports have been referenced in interactions with non-U.S. regulators, health technology assessment bodies, and other stakeholders because they provide publicly available descriptions of patient priorities developed through structured engagement processes. In this way, a single PFDD meeting may support patient-centered discussions across multiple jurisdictions and stages of the product lifecycle, extending the impact of the patient perspectives captured during the meeting.

PFDD meetings have also influenced how organizations approach patient engagement internally. Publicly available PFDD outputs have informed patient-centered development frameworks, evidence-generation planning activities, and cross-functional decision-making processes involving clinical development, regulatory affairs, patient engagement, and other functions. These efforts have encouraged more systematic approaches to incorporating patient perspectives throughout the medical product lifecycle and reinforced the value of patient experience data in development planning.

At the same time, BIO recognizes the importance of ensuring that PFDD outputs continue to reflect the diversity and evolving experiences of patient communities. As standards of care advance and treatment landscapes change, patient priorities and expectations may also evolve. For this reason, Voice of the Patient reports are often viewed as valuable reference points that may benefit from periodic reassessment and supplementation through additional patient engagement activities. Continued efforts to promote inclusivity and maintain the relevance of patient experience data will help maximize the long-term utility of these resources.

Conclusion:

BIO appreciates FDA's continued leadership in advancing patient-focused drug development and recognizes the important role PFDD meetings have played in elevating the patient voice throughout the healthcare ecosystem. Experience across therapeutic areas demonstrates that PFDD meetings have become an important mechanism for incorporating patient perspectives into research, development, and regulatory discussions. The resulting Voice of the Patient reports and other meeting outputs continue to serve as valuable resources that support patient-centered research, development, and regulatory discussions.

BIO looks forward to continued collaboration with FDA to build upon these efforts and further advance the meaningful incorporation of patient perspectives throughout the medical product lifecycle. Continued engagement among patients, patient organizations, industry, regulators, and other stakeholders will be critical to ensuring that patient experience data remain relevant, informative, and capable of supporting the development of therapies that address outcomes most important to patients.