December 30, 2025

Mr. Andrew Kish
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Center for Drug Evaluation and Research
U.S. Food and Drug Administration
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Silver Spring, MD 20903

Ms. Pamela Acero
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Ms. Emily Ewing
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Ms. Sonday Kelly
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Dear Mr. Kish, Ms. Ewing, Ms. Acero, and Ms. Kelly,

On behalf of the undersigned organizations representing individuals living with serious acute and chronic health conditions across the U.S., we write to provide the below feedback for the FDA's consideration as part of the Prescription Drug User Fee Act (PDUFA) VIII Stakeholder Consultation Meetings series.

We are truly pleased to see FDA's dedication in engaging patient communities through the stakeholder consultation meetings to inform negotiations between the Agency and industry. In an effort to share clear, timely input for the Agency's consideration, our organizations have come together to provide consolidated feedback on five shared key priorities for the PDUFA VIII negotiations:

- Refining biomarker and endpoint development outside of specific applications, including through the Drug Development Tool (DDT) qualification process;
- Advancing patient-centric drug and biologic development;
- Ensuring an adequately resourced FDA;
- Incorporating scientific advancements into regulatory practice; and
- Promoting consistency and transparency.

Refining Biomarker and Endpoint Development Outside of Specific Applications, including through the Drug Development Tool (DDT) Qualification Process

To promote greater development of biomarkers and endpoints needed to support therapeutic development for patients with unmet medical needs, we encourage FDA to:

- Conduct an assessment of the DDT qualification program, including an evaluation of how FDA is using the external expert provision and soliciting feedback from stakeholders;

- Implement performance goals for the program in a way that increases timely and meaningful feedback, including structured engagement with FDA therapeutic experts relevant to the subject of the application; and
- Directly allocate staff resources to the specific divisions overseeing each accepted application to the qualification program.

Advancing Patient-Centric Drug and Biologic Development

To build on the successes of the Patient-Focused Drug Development (PFDD) program and other Agency activities aimed at engaging patients in the drug development process, we propose that FDA:

- Develop a more continuous process for patient engagement, including earlier and more dynamic engagement, in order to better understand patient perspectives on burden of disease and risk tolerance, as well as patient preferences;
- Ensure that engagement with patient groups, including PFDD and Externally-led PFDD meetings, provides an opportunity for FDA to reflect and share learnings;
- Develop a process for meetings that facilitate timely, ongoing scientific exchange with patient groups, including development of patient reported outcomes and other endpoints, outside of the application review process;
- Direct resources towards additional guidances to expand upon existing PFDD resources;
- Provide continuous public feedback on the types of patient experience data submitted in support of applications and how the agency utilizes such information; and
- Secure sufficient resources and advanced planning to support the holding of advisory committee meetings, which provide a critical opportunity for patients and consumers to interact with experts to help ensure their viewpoints are considered by FDA.

Ensuring an Adequately Resourced FDA

As FDA staffing and resourcing are integral to the success of Agency efforts aimed at addressing scientific and regulatory challenges, we ask that FDA:

- Ensure that pilots and programs outlined above are adequately resourced to facilitate their success; and
- Adequately resource and support the Rare Disease Innovation Hub so that it can provide a forum for more fulsome patient engagement, drive regulatory efficiencies, develop methodological guidances for strategies to support rare disease drug development, and promote greater alignment across review staff.

Incorporating Scientific Advancements into Regulatory Practice

To facilitate greater patient benefit from transformative scientific advancements like cell and gene therapy and artificial intelligence (AI), we encourage FDA to:

- Develop additional disease-specific guidances focused on cell and gene therapies;
- Advance a more fit for purpose approach to the manufacturing of cell and gene therapies in order to help make the development of high-quality products more feasible for smaller populations; and

- Support an evidence- and risk-based approach to the adoption and reliance on AI in diagnostics and digital tools.
- Prioritize the qualification of endpoints derived from continuous monitoring technologies that capture real-world patient experience and treatment burden, such as Time in Range for diabetes, which better reflect daily disease management than traditional laboratory-based measures.

Promoting Consistency and Transparency

As consistency and transparency are key to increasing regulatory clarity and accelerating innovations for patients in need, we urge FDA to:

- Invest in systems-based approaches to advance product development that can be adopted across therapeutic areas;
- Provide public quarterly reporting of staff gains, losses, and recusals at the division level;
- Continue public meetings with patient and consumer groups through the implementation of the PDUFA commitments; and
- Ensure that the refinements outlined in this letter are approached in a cross-center manner to promote consistency and alignment across review offices and centers as much as scientifically justified. Such alignment may also lead to further efficiencies in drug development and review.

We appreciate FDA's continued commitment to engaging patient communities in the PDUFA reauthorization process and look forward to continued dialogue with the Agency on these and other critical topics as negotiations progress. For questions related to this letter, please contact Michelle Adams with Leavitt Partners at michelle.adams@leavittpartners.com.

Sincerely

Breakthrough T1D

diaTribe

Friends of Cancer Research

Muscular Dystrophy Association

National Organization for Rare Disorders