June 13, 2022

The Honorable Patty Murray
Chair
Senate HELP Committee
428 Senate Dirksen Office Building
Washington, DC 20510

The Honorable Richard Burr
Ranking Member
Senate HELP Committee
428 Senate Dirksen Office Building
Washington, DC 20510

The Honorable Frank Pallone
Chair
House Energy and Commerce Committee
2125 Rayburn House Office Building
Washington, DC 20515

The Cathy McMorris Rodgers
Ranking Member
House Energy and Commerce Committee
2322 Rayburn House Office Building
Washington, DC 20515

RE: Addressing Patient Experience Data in PDUFA VII

Dear Chairwoman Murray, Ranking Member Burr, Chairman Pallone, and Ranking Member McMorris Rodgers:

Thank you for the House Energy and Commerce and the Senate HELP Committees efforts to advance legislation, H.R. 7667 Food and Drug Amendments Act of 2022 and S. 4348 Food and Drug Administration Safety and Landmark Advancements (FDASLA) Act respectively, to reauthorize FDA user fee programs in a timely manner. We appreciate the thoughtfulness that the Committees have put into updating FDA policies to ensure that patients have access to safe and effective drugs as quickly as possible.

However, we write to express our concern and disappointment that neither bill contains important proposals to strengthen the patient voice in drug development. Both Committees have a long history of working with the patient advocacy sector and FDA to create a process by which the patient voice can be heard and considered during the development and review of a new therapy. When enacted into law in 2016, for example, the 21st Century Cures Act included landmark provisions to define patient experience data (PED), develop guidances for PED collection and analysis, and drive transparency around whether a sponsor submitted PED. These elements working together have significantly advanced the patient-focused drug development (PFDD) field. A June 2021 independent report required by Congress and commissioned by the FDA recognized FDA's commitment to advancing PFDD, but also specifically analyzed whether and how FDA uses PED in applications and recommended that FDA provide more information to applicants, patients, caregivers, and other stakeholders about how FDA uses patient experience data in regulatory decision-making.[1]

We believe it is time for Congress and the FDA to build on this momentum to not only ensure that useful PED is developed and submitted, but also that it is applied consistently across the agency during the review process. The robust investments being made in PED by patient communities and sponsors alike must be coupled with an open and vigorous response by the FDA.

As the Committees advance the PDUFA reauthorization, we urge you to continue your legacy of supporting the patient voice in therapy development by including language ensuring FDA discloses how it uses PED in the review process. This balance between rigor in developing PED and in applying it to the core agency decision-making is a vital compact that only Congress can ensure.

[1] Eastern Research Group, Inc. *Assessment of the Use of Patient Experience Data in Regulatory Decision-Making.* June 18, 2021. https://www.fda.gov/media/150405/download

Thank you for your consideration of these views. Please contact Ryan Fischer, Parent Project Muscular Dystrophy at ryan@parentprojectmd.org with any questions.

Sincerely,

Alstrom Syndrome International
Barth Syndrome Foundation
Best Day Ever Foundation
COPD Foundation
Cure CMD
Cure HHT
Cure Sanfilippo Foundation
Dravet Syndrome Foundation
Dup15q Alliance
Emily's Entourage
EveryLife Foundation for Rare Diseases
Foundation for Prader-Willi Research
Global Liver Institute
International Pemphigus Pemphigoid Foundation
Kindness Over Muscular Dystrophy
Lupus Foundation of America
National Ataxia Foundation
National Eczema Association
National Psoriasis Foundation
NBIA Disorders Association
Organic Acidemia Association
Parent Project Muscular Dystrophy
Phelan-McDermid Syndrome Foundation (PMSF)
RUNX1 Research Program
Ryan’s Quest
SYNGAP1 Foundation
The ALS Association
TSC Alliance
US COPD Coalition
UsAgainstAlzheimer's
UsAgainstAlzheimer's Action
Usher 1F Collaborative
Wiskott-Aldrich Foundation
Zack Heger Foundation