August 1, 2022

The Honorable Patty Murray
Chair
Committee on Health, Education, Labor & Pensions
United States Senate
Washington, D.C. 20510

The Honorable Richard Burr
Ranking Member
Committee on Health, Education, Labor & Pensions
United States Senate
Washington, D.C. 20510

The Honorable Frank Pallone
Chair
Committee on Energy and Commerce
United States House of Representatives
Washington, D.C. 20515

The Honorable Cathy McMorris Rodgers
Ranking Member
Committee on Energy and Commerce
United States House of Representatives
Washington, D.C. 20515

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

The 99 undersigned organizations, representing patients with rare diseases and other acute or chronic health conditions, urge you to resume negotiations to develop a comprehensive FDA user fee package. Our organizations are deeply concerned about the impact that a delay in passage of this legislation will have on the FDA's ability to conduct the timely review of critical products that our patients need but believe there are

other important policies that must be considered as part of the user feel reauthorization process and urge you to quickly develop robust, consensus legislation.

As you know, the Senate HELP Committee favorably reported the Food and Drug Administration Safety and Landmark Advancements (FDASLA) Act (S. 4348) out of Committee with a bipartisan 13-9 vote last month. In June, the U.S. House of Representatives considered and passed the Food and Drug Amendments of 2022 (H.R. 7667) with a bipartisan vote of 392-28. In addition to the provisions that would reauthorize FDA's user fee programs relating to prescription drugs, medical devices, biosimilars, and generic drugs, both the House and Senate bills contain additional provisions that would make necessary changes and improvements to the Federal Food, Drug, and Cosmetic Act that would ultimately benefit the patients our organizations represent. For example, both S. 4348 and H.R. 7667 would strengthen FDA's accelerated approval pathway to ensure that patients and their providers can continue to have confidence in the safety and effectiveness of drugs approved under the pathway. Furthermore, both S. 4348 and H.R. 7667 contain provisions to improve timely patient access to generic drugs and biosimilars.

Additionally, the House and the Senate bills each have distinct provisions that warrant cross-chamber consideration. For instance, H.R. 7667 includes provisions that would go a long way toward ensuring increased representation of diverse and underserved populations in the clinical trials supporting FDA-approved drugs and medical devices, but similar provisions are currently not in S. 4348. Conversely, S. 4348 includes provisions to improve FDA's oversight of the infant formula and medical food market to ensure continuous supplies of infant formula and medical foods are available that were not in the House-passed bill.

Our organizations strongly believe Congress should capitalize on the user fee reauthorization process to consider and enact the additional policies that would have little chance of passage as stand-alone bills, just as it has every five years since 1992. To pass a "clean" user fee package would be to walk away from an opportunity to make critical improvements in our nation's system for overseeing medical products. We urge you to immediately work together to blend your respective bills and continue Congress' longstanding tradition of passing strong, bipartisan FDA legislation to the benefit of all Americans. For more information, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs for the National Organization for Rare Disorders, at HRoss@rarediseases.org.

Thank you for your consideration,

National Organization for Rare Disorders
Achalasia Awareness Organization
Acid Maltase Deficiency Association (AMDA)
ADNP Kids Research Foundation
Allergy Asthma & Immunology Relief
ALS Association
American Kidney Fund
Angelman Syndrome Foundation
Arthritis Foundation
Association for Creatine Deficiencies
Avery's Hope
Born a Hero, Research Foundation
California Coalition for PKU and Allied Disorders
Charcot Marie Tooth Association
Children's Tumor Foundation
Children's Cancer Cause
Cholangiocarcinoma Foundation
Chondrosarcoma CS Foundation
Choroideremia Research Foundation
Congenital Hyperinsulinism International
Cure CMD
Cure HHT

Cure VCP Disease, Inc.
Cutaneous Lymphoma Foundation
Cystic Fibrosis Research Institute (CFRI)
Dreamsickle Kids Foundation, Inc
Dup15q Alliance
Epilepsy Foundation
Fabry Support & Information Group
FACES: The National Craniofacial Association
Fibromuscular Dysplasia Society of America
Free ME from Lung Cancer
Friedreich's Ataxia Research Alliance
Gaucher Community Alliance
Glut1 Deficiency Foundation
Gorlin Syndrome Alliance
GRIN2B Foundation
HCU Network America
Hemophilia Federation of America
Hepatitis B Foundation
Hermansky-Pudlak Syndrome Network
Hydrocephalus Association
IGA Nephropathy Foundation
Immune Deficiency Foundation
International Autoimmune Encephalitis Society
International Foundation for AiArthritis
International Pemphigus Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation
Juju and Friends CLN2 Warrior Foundation
Mississippi Metabolics Foundation
MLD Foundation
MSA Coalition
MSUD Family Support Group
Muscular Dystrophy Association
National Ataxia Foundation
National Brain Tumor Society
National MALS Foundation
National Multiple Sclerosis Society
National PKU Alliance
National PKU News
National Scleroderma Foundation
National Spasmodic Dysphonia Association
Neuromuscular Disease Foundation
No Stomach For Cancer
NTM Info & Research
Organic Acidemia Association
Pheo Para Alliance
PRISMS
Pulmonary Fibrosis Foundation
RASopathies Network USA
Reflex Sympathetic Dystrophy Syndrome Association
Remember The Girls
RETpositive
Sarcoma Foundation of America
SATB2 Gene Foundation
Sickle Cell Reproductive Health Education Directive
SLC6A1 Connect
Spina Bifida Association
SSADH Association
STXBP1 Foundation
Superficial Siderosis Research Alliance
SYNGAP1 Foundation
TANGO2 Research Organization
TargetCancer Foundation
Tatton Brown Rahman Syndrome Community
Team Telomere
The AKU Society of North America
The Association for Frontotemporal Degeneration (AFTD)
The Desmoid Tumor Research Foundation
The E.WE Foundation
The Global Foundation for Peroxisomal Disorders
The LAM Foundation
The Leukemia & Lymphoma Society
The Life Raft Group
The Patient Story
The RYR-1 Foundation
Turner Syndrome Society of the United States
Vasculitis Foundation
Wilson Disease Association

CC: The Honorable Charles E. Schumer, Majority Leader, U.S. Senate
The Honorable Mitch McConnell, Minority Leader, U.S. Senate
The Honorable Nancy Pelosi, Speaker of the House, U.S. House of Representatives
The Honorable Kevin McCarthy, Minority Leader, U.S. House of Representatives