
November 14, 2022

The Honorable Charles E. Schumer
Majority Leader
United States Senate
Washington, D.C. 20150

The Honorable Mitch McConnell
Minority Leader
United States Senate
Washington, D.C. 20515

The Honorable Nancy Pelosi
Speaker of the House
United States House of Representatives
Washington, D.C. 20515

The Honorable Kevin McCarthy
Minority Leader
United States House of Representatives
Washington, D.C. 20515

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 Congressional Leadership,

The 126 undersigned organizations, representing or treating patients impacted by rare diseases and other acute or chronic health conditions, urge you to include a full five-year reauthorization of the programs listed in Section F, Title V of H.R. 6833 ('Title V programs'), of the Continuing Appropriations and Ukraine Supplemental Appropriations Act of 2023 and to include important reforms to the Federal Food, Drug and Cosmetic Act (FFDCA) in any end-of-year legislative package under development. Provisions to strengthen the accelerated approval pathway, clarify and codify the scope of orphan drug exclusivity, and expand clinical trial diversity have broad bipartisan support and would directly benefit the rare disease community, but were left out of H.R. 6833. Our organizations are deeply concerned about the impact a delay in the long-term authorization - or a lapsed authorization of the Title V programs - and a failure to address the necessary legislative changes referenced above will have on patient access to critical, often life-saving products.

Our organizations are grateful H.R 6833 included a full five-year reauthorization of several critical FDA user fee programs, which are vital to help ensure patients gain access to essential therapies and diagnostic tools in a timely manner. However, we are alarmed that Congress included only short-term reauthorizations, through December 16, 2022, of the Orphan Products Grants Program, the Best Pharmaceuticals for Children program, and many other critical programs listed in Title V. These programs have a history of strong, bipartisan support, and have been instrumental for rare disease product development. The Orphan Products Grants Program, for instance, has supported rare disease product development since 1983 and facilitated the approval of more than 80 medical products.[1] Similarly, the Best Pharmaceuticals for Children program has been instrumental in closing knowledge gaps about the safe and effective use of pharmaceuticals in pediatric populations, including generating clinical evidence supporting the safe use of common medications to treat serious medical conditions such as seizures and infections in young children.[2] The Title V programs also include the Humanitarian Device Exemptions Program and the Pediatric Device Consortia Grants Program, which provide vital incentives to facilitate the development of medical devices for pediatric populations and other small populations[3]. Without a full five-year authorization, many of these Title V programs would either end or risk being significantly disrupted, doing a tremendous disservice to our patient communities.

We are equally concerned that by passing "clean UFAs," Congress left rare disease patients behind and missed an important opportunity to advance additional critical and timely improvements to our nation's system for overseeing medical products that would have historically been paired with UFA reauthorizations. For example, provisions to strengthen the FDA's accelerated approval pathway to ensure patients and their providers can continue to have confidence in the safety and effectiveness of drugs approved under the pathway have strong bipartisan support and were included in both the latest version of the Food and Drug Administration Safety and Landmark Advancements (FDASLA) Act (S. 4348) and the House-passed Food and Drug Amendments of 2022 (H.R. 7667). Additionally, both bills included language to clarify the intent of the Orphan Drug Act and codify the FDA's long-standing interpretation of how to appropriately award orphan drug exclusivity so that it remains an effective incentive to drive continued research into safe and effective treatments for harder to study patient

[1]https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/orphan-products-grants-program

[2] https://www.nichd.nih.gov/newsroom/news/083122-pediatric-labeling-diazepam-clindamycin

[3] https://www.fda.gov/media/74307/download

populations. Furthermore, both S. 4348 and H.R. 7667 contain provisions to improve timely patient access to generic drugs and biosimilars important to supporting rare disease patients access to affordable therapies.

The House and the Senate bills also included distinct provisions that have broad support and 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 clinical trials, although similar provisions were not in S. 4348. Conversely, S. 4348 includes crucial provisions to improve the FDA's oversight of the infant formula and medical food market to ensure continuous supplies of infant formula and medical foods are available, though these provisions were not in H.R. 7667. Our organizations believe that these changes and improvements to the FFDCA are necessary and would ultimately benefit the patients our organizations represent and serve.

We urge Congress to include a full, five-year reauthorization of the critical programs in Section F, Title V of H.R. 6833 and ensure critical changes to the FFDCA are included in any end of year legislative package. 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
Abetalipoproteinemia and Related Disorders Foundation
Acid Maltase Deficiency Association (AMDA)
ADNP Kids Research Foundation
Adrenal Insufficiency United
AiArthritis
AKU Society of North America
Alpha-1 Foundation
Alport Syndrome Foundation
ALS Association
American Behcet's Disease Association (ABDA)
American Kidney Fund
American Porphyria Foundation
Angelman Syndrome Foundation
APBD Research Foundation
APS Foundation of America, Inc
Arthritis Foundation
Asbestos Disease Awareness Organization
Association for Creatine Deficiencies
BCM Families Foundation
Boston Children's Hospital
CACNA1A Foundation
CDH International
Charcot-Marie-Tooth Association
Child Neurology Foundation
Children's Hospital of Philadelphia
Children's Tumor Foundation
Children's Wisconsin
Cholangiocarcinoma Foundation
Chondrosarcoma CS Foundation, Inc.
Choroideremia Research Foundation
Cincinnati Children's Hospital Medical Center
Coalition to Cure Calpain 3
Columbia University
Congenital Hyperinsulinism International
Conquering Gyrate Atrophy
CSNK2A1 Foundation
Cure CMD
Cure HHT
Cure Rare Disease
CURED Campaign Urging Research for Eosinophilic Diseases
Cutaneous Lymphoma Foundation
Cystic Fibrosis Research Institute
Dup15q Alliance
Epilepsy Foundation
Fabry Support & Information Group
FACES: The National Craniofacial Association
Fibromuscular Dysplasia Society of America
FOD (Fatty Oxidation Disorders) Family Support Group
Foundation For Sarcoidosis Research
Free ME from Lung Cancer
Friedreich's Ataxia Research Alliance (FARA)
Gaucer Community Alliance
Glut1 Deficiency Foundation
Gorlin Syndrome Alliance

Grin2B Foundation
Hepatitis B Foundation
Hydrocephalus Association
Hypersomnia Foundation
HypoPARAthyroidism Assoc.
IgA Nephropathy Foundation
Immune Deficiency Foundation
International Pemphigus Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation
Juju and Friends CLN2 Warrior Foundation
Lymphangiomatosis & Gorham's Disease Alliance
MdDS Foundation
Mississippi Metabolics Foundation
MLD Foundation
MPN Advocacy and Education International
MSUD Family Support Group
Muscular Dystrophy Association
Myocarditis Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National MALS Foundation
National Multiple Sclerosis Society
National PKU News
National Scleroderma Foundation
Nationwide Children's Hospital
NBIA Disorders Association
Neuromuscular Disease Foundation
NR2F1 Foundation
NTM Info & Research
Oral Cancer Foundation
Organic Acidemia Association
Parent Project Muscular Dystrophy
Pheo Para Alliance
PSC Partners Seeking a Cure
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
RASopathies Network USA
Reflex Sympathetic Dystrophy Syndrome Association
Remember the Girls
RETPositive
SATB2 Gene Foundation
Sickle Cell Assn Of Texas Marc Thomas Foundation
Sickle Cell Reproductive Health Education Directive
Spina Bifida Association
SSADH Association
STXBP1 Foundation
Tatton Brown Rahman Syndrome Community
Team Telomere
The Akari Foundation
The Association of Frontotemporal Degeneration
The Global Foundation for Peroxisomal Disorders
The Life Raft Group
The Multiple System Atrophy Coalition
The National PKU Alliance
The Progeria Research Foundation
The Recurrent Respiratory Papillomatosis Foundation
The RYR-1 Foundation
The Stiff Person Syndrome Research Foundation
The Sudden Arrhythmia Death Syndromes (SADS) Foundation
TSC Alliance
Turner Syndrome Society of the United States
United Leukodystrophy Foundation
United Porphryias Association
University of Alabama at Birmingham/Children's of Alabama
UPMC Children's Hospital of Pittsburgh
Usher Syndrome Coalition
Vasculitis Foundation
VHL Alliance
Wake Up Narcolepsy
Xia-Gibbs Society, Inc.