The Honorable Patty Murray
Chair
Senate Appropriations Committee
S-128 Capitol Building
Washington, D.C. 20515

The Honorable Tom Cole
Chairman
House Appropriations Committee
H-307 Capitol Building
Washington, D.C. 20515

The Honorable Susan Collins
Ranking Member
Senate Appropriations Committee
S-128 Capitol Building
Washington, D.C. 20515

The Honorable Rosa DeLauro
Ranking Member
House Appropriations Committee
1036 Longworth House Office Building
Washington, D.C 20515

Dear Chair Murray, Ranking Member Collins, Chairman Cole and Ranking Member DeLauro,

We, the undersigned patient organizations and related stakeholders, are **writing to express our support for the inclusion of report language to establish the Food and Drug Administration (FDA) Rare Disease Center of Excellence in the Agriculture, Rural Development, Food and Drug Administration and Related Agencies Fiscal Year 2025 appropriations bill.** The report language directs the FDA to create an Intercenter Institute for Rare Diseases that will serve as a cross-cutting, capacity-building, collaborative hub for rare disease activity at the FDA.

While interest in rare disease therapy development has increased since the passage of the historic Orphan Drug Act of 1983[1], the regulatory systems we have in place struggle to meet the unique challenges and complexities inherent in rare diseases. The last 40 years have yielded tremendous progress, going from 38 approved drugs to more than 1,200 approved indications for rare diseases[2]. Despite significant scientific advancements, the rare disease community continues to face substantial obstacles in the development, review, and approval of safe and effective treatments. With over 10,000 rare diseases affecting more than 30 million Americans[3], the urgency for a streamlined and focused approach in regulatory science and review processes cannot be overstated. About 95 percent of rare disease communities still lack an FDA-approved

[1] Fermaglich LJ, Miller KL. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. Orphanet J Rare Dis. 2023 Jun 23;18(1):163. doi: 10.1186/s13023-023-02790-7. PMID: 37353796; PMCID: PMC10290406.
[2] FDA. (n.d.). Search orphan drug designations and approvals. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm
[3] Groza, T., McMurry, J., Dawkins, H., Rath, A., Thaxon, C., Bocci, G., Joachimiak, M. P., Köhler, S., Robinson, P. N., Mungall, C., & Oprea, T. I. (2020). How many rare diseases are there? Nature Reviews Drug Discovery, 19(2), 77–78. https://doi.org/10.1038/d41573-019-00180-y

treatment[4] and significant unmet needs remain for the communities that do have an approved treatment.

Through the 21st Century Cures Act, the FDA received the authority to establish one or more Intercenter Institutes for a major disease area or areas[5]. A Rare Disease Center of Excellence would bring together the extensive rare disease expertise across the FDA in one central location. A Center of Excellence would help organize all FDA resources – such as statisticians, regulatory scientists and experts in clinical trial design for small populations – within a single structure to avoid duplication and disciplinary silos as well as to make concentrated resources available to multiple review divisions. It would recognize that despite the wide diversity in clinical symptoms and organ systems affected by rare diseases, the barriers to effective therapeutic development are similar.

A Rare Disease Center of Excellence can address rare disease regulatory challenges. Small patient populations create challenges that require broad FDA expertise to address. Identifying the natural progression of disease, dispersing clinical trial sites, detecting clinically meaningful outcomes, and designing alternative clinical trials are all common across rare disease therapy development programs, but can be unique issues for a review team evaluating a rare disease therapy. In addition, the dispersion of rare disease experts across the entire FDA limits the ability to share best practices on how to address these challenges.

Time is the most precious commodity for the rare disease community. Each time a promising therapeutic target faces delays or demise due to the complexities in rare disease and strain on the existing regulatory infrastructure, lives are lost, investment is lost, and future scientific promise is unfulfilled. The creation of a Rare Disease Center of Excellence would not only catalyze scientific and medical breakthroughs but also offer hope to millions of Americans living with rare diseases.

Thank you for considering the needs of the rare disease patient community in the 118th Congress. The establishment of a Rare Disease Center of Excellence at FDA is a significant step forward in bridging the gap between rare disease patients and the innovative treatments urgently needed.

Should you have any questions, please reach out to Annie Kennedy with the EveryLife Foundation for Rare Diseases at akennedy@everylifefoundation.org.

Sincerely,

The EveryLife Foundation for Rare Diseases
Adult Polyglucosan Body Disease Research Foundation

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[4] National Center for Advancing Translational Sciences (NCATS). (2023). Delivering Hope for Rare Diseases. NCATS. https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf

[5] 21st Century Cures Act, H.R. 34, 114th Cong. (2015).

Alexion, AstraZeneca Rare Disease
Alliance for Patient Access
Alpha-1 Foundation
Alport Syndrome Foundation
Amicus Therapeutics
Amyloidosis Foundation
Angelman Syndrome Foundation
Association for Creatine Deficiencies
Autoinflammatory Alliance
Avery's Hope
Barth Syndrome Foundation
Biogen
Born a Hero, Research Foundation
CA Action Link for Rare Diseases (Cal Rare)
California Life Sciences
Canary Advisors LLC
Center for Patient Advocacy Leaders (CPALs)
Congenital Adrenal Hyperplasia Research, Education & Support Foundation DBA: CARES Foundation
COPD Foundation
Cure CMD
Cure GM1 Foundation
Cure HHT
Cure SMA
Cure VCP Disease
CureARS
CureDuchenne
CureLGMD2i Foundation
CureSHANK
CureSPG50
Cyclic Vomiting Syndrome Association
Cystic Fibrosis Research Institute
Danny's Dose Alliance
Dravet Syndrome Foundation
EB Research Partnership
Elpida Therapeutics SPC
Every Cure
Foundation for Angelman Syndrome Therapeutics (FAST)
FD/MAS Alliance
G6pd Deficiency Foundation, Inc.
Galactosemia Foundation

Gaucher Community Alliance
Gene Giraffe Project
Global Genes
Harmony Biosciences
HCU Network America
Hereditary Angioedema Association
Huntington's Disease Society of America
Hyman, Phelps & McNamara, PC
Immune Deficiency Foundation
Juju and Friends CLN2 Warrior Foundation
Lennox-Gastaut Syndrome (LGS) Foundation
Leukodystrophy Newborn Screening Action Network
LGMD2D.org
Lipodystrophy United
Little Hercules Foundation
Little Miss Hannah Foundation
Lupus and Allied Diseases Association, Inc.
Mahzi Therapeutics
Mission MSA
Mississippi Metabolics Foundation
MLD Foundation
Muenzer MPS Research & Treatment Center
Myasthenia Gravis Foundation of America (MGFA)
Myositis Support and Understanding
National Fragile X Foundation
National Leiomyosarcoma Foundation
National MPS Society
National PKU Alliance
National Society of Genetic Counselors
NBIA Disorders Association
NTM Info & Research
Organic Acidemia Association
Parent Project Muscular Dystrophy
Phoenix Nest
Pompe Alliance
Project Alive
PWSA | USA - Prader-Willi Syndrome Association
Rare and Undiagnosed Network (RUN)
Remember The Girls
Rhythm Pharmaceuticals
Sarcoidosis of Long Island

SCAD Alliance (spontaneous coronary artery dissection)
SCID Angels For Life Foundation
Siegel Rare Neuroimmune Association
SLC6A1 Connect
Stealth BioTherapeutics
STXBP1 Foundation
SynGAP Research Fund, Inc.
Team Titin
TESS Research Foundation
The Bluefield Project to Cure Frontotemporal Dementia
The Ehlers-Danlos Society
The Guthy-Jackson Charitable Foundation
The LAM Foundation
The Oxalosis and Hyperoxaluria Foundation
Travere Therapeutics
Undiagnosed Diseases Network Foundation
United Mitochondrial Disease Foundation
United MSD Foundation
Wiskott-Aldrich Foundation
Wylder Nation Foundation