



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<sup>1</sup>, 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<sup>2</sup>. 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<sup>3</sup>, 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

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<sup>1</sup> 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.

<sup>2</sup>FDA. (n.d.). Search orphan drug designations and approvals. <https://www.accessdata.fda.gov/scripts/opdlisting/ood/listResult.cfm>

<sup>3</sup> 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<sup>4</sup> 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<sup>5</sup>. 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 118<sup>th</sup> 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](mailto:akennedy@everylifefoundation.org).

Sincerely,

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

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

<sup>5</sup> 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  
Traverse Therapeutics  
Undiagnosed Diseases Network Foundation  
United Mitochondrial Disease Foundation  
United MSD Foundation  
Wiskott-Aldrich Foundation  
Wylder Nation Foundation