

November 12, 2015

The Honorable Lamar Alexander, Chairman
Senate Committee on Health, Education,
Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray, Ranking Member
Senate Committee on Health, Education
Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Chairman Alexander and Ranking Member Murray:

The undersigned organizations, representing millions of Americans living with a rare disease, write today to express our strong support for the *Advancing Targeted Therapies for Rare Diseases Act of 2015* (S. 2030). If enacted, this legislation could greatly accelerate the pace of therapeutic development for rare diseases and their sub-populations by streamlining the regulatory review process, without compromising safety and efficacy standards.

This legislation clarifies FDA's existing authority to leverage data previously used in the approval of a targeted drug when approving a new therapy that "incorporates or utilizes the same or similar genetically targeted technology, or the same variant protein targeted technology".

This authority is critically important to accelerating the development of treatments and cures for the numerous devastating rare diseases or subsets of rare diseases that otherwise have little hope of a treatment or cure due to their extremely small population size. By clarifying that the FDA is allowed to use previously accepted data in certain circumstances, this provision will help facilitate the development and approval of therapies for these diseases.

This bill does not jeopardize intellectual property or patent protections, as only the sponsor that generated the original data may leverage that same data for future uses, unless the sponsor grants permission otherwise. Additionally, this legislation does not bind the FDA in its review processes, and instead reiterates existing flexibility in the data they are permitted to accept.

This bill is supported throughout the rare disease stakeholder community, and we urge the Senate Committee on Health, Education, Labor and Pensions to include this bill in the Senate Innovation for Healthier Americans Initiative.

Thank you for your continued dedication to the rare disease community, and we look forward to working with you to ensure this provision is enacted.

Sincerely,

AKU Society of North America
Alpha-1 Foundation
ALS Association
Alternating Hemiplegia of Childhood Foundation
American Behcet's Disease Association (ABDA)
American Brain Tumor Association
American Multiple Endocrine Neoplasia Support (AMEN Support)
Association for Frontotemporal Degeneration (AFTD)
Association of Gastrointestinal Motility Disorders, Inc. (AGMD)
Association for Glycogen Storage Disease
Batten Disease Support and Research Association
BCC Nevus Syndrome Life Support Network
BRBN Alliance
Children's PKU Network
Cholangiocarcinoma Foundation
Chromosome Disorder Outreach Inc.
Cicatricial Alopecia Research Foundation
Council For Bile Acid Deficiency Diseases
CureCADASIL (CADASIL Association Inc.)
CureCMD
Cure HHT
Cutaneous Lymphoma Foundation
The Desmoid Tumor Research Foundation, Inc.
Dystonia Advocacy Network
Dystonia Medical Research Foundation
dystrophic epidermolysis bullosa research association of America (debra of America)
The Erythromelalgia Association
Everylife Foundation for Rare Diseases
Foundation for Ichthyosis & Related Skin Types, Inc.
Foundation for Prader-Willi Research
Foundation to Eradicate Duchenne (FED)
Friedreich's Ataxia Research Alliance (FARA)
GBS/CIDP Foundation International
The Global Foundation for Peroxisomal Disorders
The Guthy – Jackson Charitable Foundation
Hermansky-Pudlak Syndrome Network Inc.
Histiocytosis Association
HLRCC Family Alliance
The Huntington's Disease Society of America
HypoPARathyroidism Association
Immune Deficiency Foundation
Indian Organization for Rare Disorders
The International Advocate for Glycoprotein Storage Diseases
International FOP Association
International Foundation for CDKL5 Research
International Myeloma Foundation

International Pemphigus and Pemphigoid Foundation (IPPF)
International WAGR Syndrome Association
Jack McGovern Coats' Disease Foundation
Kennedy's Disease Association
LAL Solace
The Life Raft Group
Lymphangiomatosis & Gorham's Disease Alliance
The Marfan Foundation
MEBO Research
MitoAction
Moebius Syndrome Foundation
The Morgan Leary Vaughan Fund
Mucopolidosis Type IV Foundation
Muscular Dystrophy Association (MDA)
The Myositis Association
National Adrenal Diseases Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Eosinophilia Myalgia Syndrome Network
National Lymphedema Network (NLN)
National MPS Society
National Organization for Rare Disorders (NORD)
National PKU Alliance
National Spasmodic Dysphonia Association
National Tay-Sachs & Allied Diseases Association, Inc. (NTSAD)
NBIA Disorders Association
NephCure Kidney International
Neuroendocrine Tumor Research Foundation
Neurofibromatosis Network
The Oley Foundation
Organic Acidemia Association
Osteogenesis Imperfecta Foundation
Oxalosis and Hyperoxaluria Foundation
Parent Project Muscular Dystrophy (PPMD)
Parents and Researchers Interested in Smith-Magenis Syndrome (PRISMS)
PKD Foundation
Prader-Willi Syndrome Association (USA)
PRP Alliance
Pulmonary Hypertension Association
RASopathies Network USA
Rett Syndrome Research Trust
Scleroderma Foundation
Spastic Paraplegia Foundation
Sturge-Weber Foundation
Tarlov Cyst Disease Foundation
Tuberous Sclerosis Alliance

United Leukodystrophy Foundation
The United Mitochondrial Disease Foundation
US Hereditary Angioedema Association
Vasculitis Foundation
VHL Alliance
Williams Syndrome Association
Wilson Disease Association
Worldwide Syringomyelia & Chiari Task Force
XLH Network

For additional information, contact:

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CC: Members of the Senate Health, Education, Labor and Pensions (HELP) Committee