

April 9, 2012

The Honorable Edward Markey
U.S. House of Representatives
2108 Rayburn House Office Building
Washington, DC 20515

The Honorable Tom Marino
U.S. House of Representative
410 Cannon House Office Building
Washington, DC 20515

The Honorable Cliff Stearns
U.S. House of Representatives
2306 Rayburn House Office Building
Washington, DC 20515

Dear Representatives Markey, Marino, and Stearns:

The undersigned organizations, representing millions of Americans with rare and genetic conditions and those invested in the review of rare disease treatments, strongly support the passage of the bipartisan Expanding and Promoting Expertise in Review of Rare Treatments (EXPERRT) Act, S. 2281, and commend you for your commitment to the development of treatments for this vulnerable population.

The Food and Drug Administration (FDA) currently confronts an exciting but daunting scientific environment. As we reap the benefits of the mapping of the human genome and the creation of innovative models for advancing drug development, treatments are being developed for more rare diseases and small subsets of diseases than ever before.

The challenge for drug reviewers is obtaining access to the most specialized, up-to-date information on the rare disease treatments they are evaluating. There are thousands of rare diseases, and reviewers are stretched to have proficiency in every rare disease treatment they are called on to evaluate. Among the difficult issues they must address are the design of clinical trials for rare diseases and subtypes of diseases, the burden of rare diseases and the unmet medical needs of those with rare diseases, benefit and risk assessment for rare diseases, and the demographics of rare disease patient populations.

The complexity and diversity of applications for rare disease therapies suggest that the agency would benefit from increased consultation with external experts on rare diseases, to ensure that those evaluating treatments have the help they need to move applications through the review process efficiently, effectively, and without delay.

The EXPERRT Act addresses this need by facilitating consultation with external experts like academic researchers, health professional organizations, patient advocates and care providers about issues related to rare diseases. The FDA has the authority to consult with external experts currently, and the EXPERRT Act will encourage that practice, which will be of enhanced importance in an age of targeted therapies for rare diseases. The EXPERRT Act would help make this best practice a standard practice.

Again, we applaud your commitment to the rare disease community and wish to convey our strong support for the EXPERRT Act. We look forward to working with you to secure passage of this bill that promises to enhance the review of treatments for millions of Americans.

Sincerely,

Alpha-1 Association
Alpha-1 Foundation
Alstrom Syndrome International
American Sleep Apnea Association
Amyloidosis Support Groups Inc
ARPKD/CHF Alliance
Batten Disease Support and Research Assn.
Benign Essential Blepharospasm Research Foundation
CADASIL Together We Have Hope
CFC International
Children's Interstitial Lung Disease (chILD) Foundation
Coalition of Heritable Disorders of Connective Tissue
Congenital Adrenal hyperplasia Research, Education and Support
COPD Foundation
Cystic Fibrosis Foundation
Detroit Medical Reserve Corps
Digestive Disease National Coalition
Dystonia Medical Research Foundation
Everylife Foundation for Rare Diseases
Facing Our Risk of Cancer Empowered (FORCE)
Faster Cures: The Center for Accelerating Medical Solutions
Foundation Fighting Blindness
Genetic Alliance
Hereditary Hemorrhagic Telangiectasia (HHT) Foundation
International
Hermansky-Pudlak Syndrome Network Inc.
Huntington's Disease Society of America
International Society of Nurses in Genetics
Lymphatic Research Foundation
MLD Foundation
Moebius Syndrome Foundation
Narcolepsy Network, Inc.
National Fabry Disease Foundation
National Foundation for Ectodermal Dysplasias
National Gaucher Foundation
National Marfan Foundation
National Organization for Rare Disorders (NORD)

National Spasmodic Dysphonia Association
National Tay-Sachs & Allied Diseases Association, Inc. (NTSAD)
NBIA Disorders Association
Parent Project Muscular Dystrophy
Parents and Researchers Interested in Smith-Magenis Syndrome
(PRISMS)
Parkinson's Action Network
Primary Ciliary Dyskinesia Foundation
Project DOCC - Delivery of Chronic Care
PTC Therapeutics
Pulmonary Fibrosis Foundation
PXE International
RARE Project
Respiratory Health Association of Metropolitan Chicago
Rett Syndrome Research Trust
Scleroderma Foundation
Tuberous Sclerosis Alliance
U.S. Hereditary Angioedema Association
VHL Family Alliance
Wilson Disease Association

Cc: Chairman Fred Upton, Committee on Energy and Commerce
Chairman Joe Pitts, Committee on Energy and Commerce, Subcommittee on Health
Ranking Member Henry A. Waxman, Committee on Energy and Commerce
Ranking Member Frank Pallone, Committee on Energy and Commerce, Subcommittee on Health