April 4, 2017

The Honorable Kamala Harris  
112 Hart Senate Office Building  
United States Senate  
Washington, D.C.  20510

Dear Senator Harris:

As members of the rare disease advocacy community in California, we write today to request that you serve as the Democratic lead sponsor of the Orphan Product Extensions Now, Accelerating Cures and Treatments (OPEN ACT).

This vital legislation would provide a needed incentive to encourage biopharmaceutical companies and innovators to repurpose existing therapies for rare disease indications. In doing so, the OPEN ACT would lead to the development of hundreds of new, safe, effective and affordable treatment options for the 1 in 10 Americans with a rare disease – nearly four million of whom reside in California.

As it stands, 95% of the 7,000 rare diseases have no FDA-approved treatment, and for many patients with progressive and deadly disease, time is running out. In fact, 30% of children diagnosed with a rare disease will not live to see their 5th birthday. Passing the OPEN ACT will be the fastest and most efficient way for rare disease patients to gain access to new treatments which will ultimately save lives.

In addition to providing new treatment options to patients, the OPEN ACT would require extensive clinical testing and research studies to gain FDA-approval for a new indication. Such clinical studies would be carried out at academic medical centers across the Nation and at the top research centers here in California, providing a substantial infusion of research dollars to undertake needed studies. This new investment would help create high-quality, research-intensive jobs that would play a key part in uncovering new therapy options for rare disease.

Thank you for your support of research and innovation here in California and across America. This support is helping save the lives of so many Americans facing the challenges of living with a rare disease.

Again, we request that you co-sponsor the OPEN ACT as the lead Democrat in the Senate. We stand ready to work with you and your staff to advocate for this important public policy priority.

Sincerely,

EveryLife Foundation for Rare Diseases, Novato  
Achalasia Awareness Organization, Los Angeles  
ADCY5.org, La Jolla  
Alpha-1 Foundation, Thousand Oaks  
Association for Creatine Deficiencies, Carlsbad  
Autoinflammatory Alliance, San Diego  
Axis Advocacy, Eagle Rock
Children's PKU Network, Encinitas
Connecting Families Urea Cycle Disorders Foundation, Murrieta
Cure CMD, International Patient Advocacy Nonprofit Organization, Torrence
CureDuchenne, Newport Beach
Cystic Fibrosis Research, Inc. (CFRI), Palo Alto
Foundation for Prader-Willi Research, Oceanside
GCAF GIST Cancer Awareness Foundation, Ramona
Global Genes, Aliso Viejo
GNE Myopathy International, Watsonville
Gold Rush Cure Foundation, Laguna Niguel
Grace Science Foundation, San Francisco
Health & Science Institute, Indian Wells
HemoAwareness Project, Sacramento
International Pemphigus & Pemphigoid Foundation, Ventura
Intractable Pain Patients United, Lancaster
Making Change For Children, Canoga Park
Myasthenia Gravis Foundation, Seal Beach
National Lymphedema Network, Berkeley
National MPS Society, Modesto
National Spasmodic Torticollis Association, Fountain Valley
NBIA Disorders Association, San Diego
Neuromuscular Disease Foundation (NDF), Beverly Hills
Neuropathy Action Foundation, Santa Ana
Parkinson's and Movement Disorder Foundation, Westminster
PROS Foundation, San Diego
Rare Genomics Institute, Downey
RASopathies Network USA, Altadena
Sick Chicks, Carona Del Mar
The Bluefield Project to Cure Frontotemporal Dementia, San Francisco
The Nicholas Conor Institute for Pediatric Cancer, San Diego