February 1, 2016

The Honorable Elizabeth Warren  
United States Senate  
Washington, D.C.  20510  

Dear Senator Warren:

On behalf of the rare disease patients and their families living in Massachusetts, the undersigned organizations are writing to request your support for bipartisan legislation that would significantly increase the number of treatments for rare diseases. With 95 percent of rare diseases having no FDA-approved treatment, it is critical that Congress enact policies that will bring aid to the nearly 30 million Americans suffering from a rare disease or condition.

We urge you to support the inclusion of S. 1421, the Orphan Product Extensions Now Accelerating Cures and Treatments Act of 2015 (OPEN ACT), in the Senate's medical research and innovation legislation being considered this fall. This bipartisan bill sponsored by Senators Amy Klobuchar (D-MN) and Orrin Hatch (R-UT) would lead to the development of hundreds of safe, effective, and affordable rare disease treatments within the next several years by incentivizing drug makers and innovators to “repurpose” already approved drugs for life-threatening rare diseases and pediatric cancers. In exchange for investing the resources to achieve an FDA-approved rare disease indication on the medicine’s label, the OPEN ACT would grant six months of market exclusivity to repurposed drug.

Requiring drug makers and innovators to obtain FDA-approval for the rare disease treatment means that patients can have confidence that the medicines they are taking are thoroughly tested for safety and effectiveness, and that researchers have studied and determine dosage levels. This would decrease unmonitored off-label use and would clear the way for insurance coverage.

As the Senate Health, Education, Labor and Pensions Committee, as well as the full Senate, considers medical research legislation, we would ask that you join the more than 150 rare disease organizations across the country in supporting the inclusion of the OPEN ACT, S. 1421 in this legislative package.

Sincerely,

National Organization for Rare Disorders (NORD), Quincy  
The Coalition Against Pediatric Pain (TCAPP), Medfield  
Rare Disease United Foundation, Attleboro  
Sophia's Fund, Marlborough  
Choroideremia Research Foundation, Inc., Springfield  
CureDuchenne, Cambridge  
TargetCancer Foundation, Cambridge  
National Tay-Sachs & Allied Diseases Association, Boston  
Amyloidosis Research Consortium, Lincoln