August 16, 2016

The Honorable Chuck Schumer
United States Senate
Washington, D.C.  20510

The Honorable Kirsten Gillibrand
United States Senate
Washington, D.C.  20510

Dear Senator Schumer and Senator Gillibrand:

As the Senate considers medical innovation legislation this year, we request that you support the bipartisan Orphan Product Extensions Now Accelerating Cures and Treatments Act (OPEN ACT), which 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 aid the nearly 30 million Americans suffering from a rare disease or condition.

The bipartisan OPEN ACT (S. 1421), 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.

Repurposing is the most cost-efficient and expedient method to bring new therapy options to rare disease patients, and would actually lower the cost of orphan drugs because they would be priced at the level of the major market indications. This is in contrast to highly innovative and specialized therapies for rare disease patients whose cost may be higher than current therapy options. Furthermore, when repurposed drugs become generic, they will retain the new rare disease indications on the label for all time, which again would provide a lower-cost, long-term, FDA-approved treatment option for rare disease patients.

In addition to new treatment options, this bill would require extensive clinical testing and research studies in order to gain FDA-approval for a new indication. Such clinical studies would be carried out at academic medical centers across the Nation, providing a substantial infusion of research dollars to undertake needed studies. This new investment would create high-quality, research-intensive jobs that would play a key part in uncovering new therapy options for rare disease patients and would complement efforts to increase NIH funding, which we support and applaud.

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, making the cost of these therapies more affordable.
As the Senate considers the medical research legislation expected this year, we would ask that you join the more than 167 patient organizations across the country in supporting the OPEN ACT, S. 1421.

Sincerely,

Hermansky-Pudlak Syndrome Network Inc., Oyster Bay
Cardio-Facio-Cutaneous (CFC) International, Vestal
Jonah's Just Begun-Foundation to Cure Sanfilippo Syndrome, Brooklyn
Adult Polyglucosan Body Disease, Brooklyn
CurePSP, Inc., NYC
Sarcoidosis of Long Island, Coram
Foundation for Sarcoidosis Research, NYC
CCHS Family Network & CCHS Foundation, Oneonta
Liddy Shriver Sarcoma Initiative, Ossining
Jeffrey Modell Foundation, NYC
Aidan Jack Seeger Foundation, Brooklyn