



Rare Disease  
Innovations Institute  
*Rare Disease Day Is Every Day*



**PDL1**  
Amplifieds

Exon20  
EGFR/HER2  
EXON 20 MUTATION GROUP

**ican**<sup>®</sup> international  
cancer  
advocacy  
network  
the virtual lifeline for proactive cancer patients



SOME 1 LIKE YOU  
PRIVATELY CONNECTING THOSE WITH HEALTH CONDITIONS



**ADNP Kids**  
Research Foundation  
Understanding **ADNP SYNDROME** / *Heismoorstel - VanDerAa Syndrome*



July 22<sup>nd</sup> 2022

Dear Senator Cantwell,

You have long been known for your strong efforts to deliver low-cost, high-quality healthcare to Washingtonians. Your focus on increasing support for rare disease patients has always made us feel that we have an advocate fighting to help us access treatments that make a critical difference in our lives. That's why we wanted to share our concerns about the Prescription Drug Pricing Reform proposal that was recently advanced in the U.S. Senate. This proposal will do irreparable harm to healthcare access for rare disease patients, and it will set our ability to have a say in our healthcare back decades.

The Congressional Budget Office (CBO) recently released an [analysis](#) that shows this legislation would lead to a 10% reduction in the number of new drugs on the market. While this number may not initially sound high, the reality is that many rare disease patients have been waiting years or decades for care solutions. Today, 95% of rare diseases have [no approved treatment](#). For patients that have few or no care options, any reduced ability to get the treatment they need is unsafe and harmful. If passed into law, the proposal will fundamentally reduce rare disease patients' ability to access care.

The engine of medical innovation that is our U.S. healthcare system has for decades solved some of the most difficult treatment challenges rare disease patients face. But the drug pricing proposal would slash much-needed investment into research and development of drugs that are desperately needed by patients like us. The proposed reforms would also reduce drug development for secondary indications,

meaning, fewer medicines will be tested and approved for therapeutic applications beyond their original purpose. This is particularly harmful to medicines that address pediatric diseases.

As a longtime voice for Washington Medicaid patients and a proponent of patient-driven care, the legislation's proposed price-setting measures should also alarm you. By picking and choosing which drugs can have their prices negotiated, Medicaid will effectively determine which drugs are more likely to be prescribed. It will put less cost-effective treatments out of reach for low-income patients as health systems will be less likely to prescribe them. Rare disease patients on Medicaid, who face much higher cost-of-care than average patients, will have reduced ability to choose the drugs that are best for their quality of life.

If the Drug Pricing Reform proposal is implemented, rare disease patients will be set back years in their ability to access care. The Pharmacy Benefit Manager Transparency Act bill you introduced with Sen. Grassley is needed now more than ever to reduce patient costs without shrinking U.S. healthcare innovation. We hope that in the weeks and months ahead, you will continue to press for its passage and find solutions that increase healthcare equity for Washington rare disease patients.

Sincerely,

Joshua Henderson, Co-founder of the Northwest Rare Disease Coalition

Carolina Sommer, CEO & Founder, Born a Hero Rare Disease Research Foundation

Tara Britt, Founder & President Rare Disease Innovations Institute

Karen Ferguson, MS, Founder, Our Stories Rx

Steven Newmark, Director of Policy and General Counsel, Global Healthy Living Foundation

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Ellen Morgan, Founder & President, Pros Foundation

Makayla Allison King, Founder, Some 1 Like You

Sandra Bedrosian-Sermone, Founder, CEO, President, ADNP Kids Research Foundation

Austin Stack, Founder and Executive Director of XLA Life