

March 30, 2015

The Honorable Fred Upton
Chairman
Committee on Energy & Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
Committee on Energy & Commerce
U.S. House of Representatives
2322A Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton and Ranking Member Pallone:

The undersigned represent biopharmaceutical innovators focused on providing life-transforming therapies to patients with severe and life-threatening rare diseases. We join with patient advocates and urge you to permanently authorize the Rare Pediatric Disease Priority Review Voucher (PRV) Program to support greater development of treatments for children suffering from rare pediatric diseases.

Despite preexisting legislation intended to accelerate rare disease therapies to market, drug development in pediatric rare diseases has been neglected. Recognizing this neglect, as part of the Food and Drug Administration Safety and Innovation Act of 2012, Congress supported the Rare Pediatric Disease PRV Program to foster drug development for rare and neglected diseases in children.

Since the passage of the Rare Pediatric Disease PRV Program, the Food and Drug Administration (FDA) has awarded three vouchers. The first voucher was awarded to a biological product approved for patients with Morquio A syndrome, a rare, severely debilitating and progressive disease that previously had no standard accepted treatment other than supportive care. The second voucher was awarded to a biological product for patients with neuroblastoma, a pediatric cancer. Notably, this product is only the third drug that has received initial FDA approval for a pediatric cancer in over 20 years. The third voucher was recently issued to the first FDA approved treatment for certain bile acid disorders that stunt growth and can result in life-threatening liver damage.

The PRV program ends in early 2016. With its termination, companies are left without a crucial incentive to invest in risky and challenging research and development efforts. A program that encourages more treatments for rare pediatric diseases is lost.

Rare disease biopharmaceutical innovators are utilizing the PRV program as intended, as evidenced by the three granted vouchers. The patient community clearly needs a program that drives biopharmaceutical companies to invest in research and development for rare pediatric diseases and provides hope to children who are suffering from these rare conditions.

We urge you to support legislation that permanently reauthorizes this critical program.

Thank you for your consideration of our views. We look forward to working with you.

Sincerely,

Adaptive Biotechnologies
Alexion Pharmaceuticals
Amicus Therapeutics
BayBio
Bayer Corporation
BioMarin
Biotechnology Industry Organization
California Healthcare Institute
CSL Behring
Genzyme
HealthCare Institute of New Jersey
Horizon Pharma
Insmad, Inc.
Lumos Pharma
Marathon Pharmaceuticals
Massachusetts Biotechnology Council
Pennsylvania Bio
Pfenex Inc.
Recordati Rare Diseases
Retrophin
Sarepta Therapeutics
Shire
Synageva BioPharma Corp.
Texas Healthcare and Bioscience Institute
Ultragenyx Pharmaceutical
Vertex Pharmaceuticals
XOMA Corporation