

KIDS CANCER

Changing the landscape of pediatric research

May 17, 2016

The Honorable Fred Upton
Chairman
Energy and Commerce Committee
U.S. House of Representatives Washington,
DC 20515

The Honorable Frank Pallone
Ranking Member
Energy and Commerce Committee
U.S. House of Representatives Washington,
DC 20515

Dear Chairman Upton and Ranking Member Pallone:

I am writing to express my concern about Strengthening Public Health Emergency Response Act of 2015, H.R.3299. I request that you not support Section 8 of this bill, to establish a new priority review voucher program.

Section 8 of this bill would directly harm American children with cancer and life threatening illnesses. It would break what is now a very effective drug development incentives program for seriously ill children: the Rare Pediatric Disease Priority Review Voucher Program, also known as the Creating Hope Act, 21 U.S.C. 360ff (Pediatric PRV). Protecting Americans from a possible risk of bioterrorism is also an important goal, but please do not pursue it at the expense of ill children.

Although the United States has a robust pharmaceutical industry, very few drugs have been developed for children with cancer. In the 20 years leading up to the passage of the Pediatric PRV, there had been only one initial FDA approval for a drug expressly for pediatric cancer, the leading cause of death by disease for children. Children's cancers are different from adult cancers and children are treated with drugs that are decades old, even as cancer research has undergone transformative leaps forward.

In 2012, Congress responded to this crisis in pediatric cancer by including in the U.S. Food and Drug Administration Safety and Innovation Act the Pediatric PRV, which was based on the Neglected Tropic Disease Priority Review Voucher program. With the passage of the Pediatric PRV, companies are now, for the first time, actively developing drugs expressly for children with cancer and in the next 10 years we will finally see newly approved life saving cancer drugs for our kids.

Of course the sweeping success of the PRV program has attracted attention and as a result you are considering a new bioterrorism PRV. However, as Dr. David Ridley of Duke University explains, expansion of PRV program will break the Pediatric PRV because the value of the voucher will plummet quickly.¹ Current pricing for vouchers can only be supported if the number of vouchers is not increased by the creation of new PRV programs. Even a small increase in the number of

¹ David B. Ridley and Stephane A. Régnier, The Commercial Market For Priority Review Vouchers,

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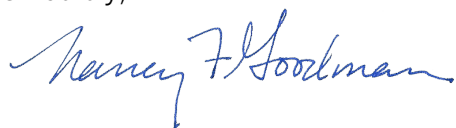
vouchers will dramatically depress the value of the Pediatric PRV -- and of every other PRV -- and undermine the incentive to which companies are now responding.

A new PRV program would be directly at the expense of children's health. It also would not be an effective incentive because the value of *all* PRVs would be depressed. The Strengthening Public Health Emergency Response Act includes other important provisions to increase development of new bioterrorism drugs. These provisions are uniquely suited for bioterrorism because they take into consideration the importance of military leadership in identifying and directing the prioritization of bioterrorism drug development. If these provisions are insufficient, they should be fixed or new incentives should be developed.

The problem is simple: if everything is a priority, then nothing is a priority.

Thank you again for protecting the Pediatric PRV and protecting American children by not supporting a new bioterrorism PRV.

Sincerely,

A handwritten signature in blue ink that reads "Nancy F. Goodman". The signature is fluid and cursive, with the first name "Nancy" and last name "Goodman" clearly legible.

Nancy Goodman
Executive Director
Kids v Cancer