

KIDS CANCER

Changing the landscape of pediatric research

March 14, 2015

Friends:

I just learned that **a third Creating Hope Act voucher** was awarded!

The FDA awarded the voucher to Asklepiion Pharmaceuticals for the approval of Cholbam to treat kids with rare bile acid synthesis disorders.

What a great way to celebrate St Patrick's day and my mother's birthday.

I am thrilled that the Creating Hope Act has fulfilled its mission to support pediatric cancer and pediatric rare disease drug development.

The first pediatric priority review voucher was awarded on February 14, 2015, for Vimizim, which treats Morquio-A syndrome. The second pediatric priority review voucher was awarded on March 10, 2015 for Unituxin, which treats a pediatric cancer, neuroblastoma.

Pursuant to the terms of the Creating Hope Act, this third voucher will start the one year period, after which the Creating Hope Act will sunset on March 14, 2016.

Kids v Cancer is working with Members of Congress to introduce a bill to permanently reauthorize the pediatric priority review voucher program of the Creating Hope Act.

But that alone is not enough. Although Creating Hope Act vouchers provide a meaningful reward for drug development expressly for pediatric cancers and other pediatric rare diseases, it is still exceedingly difficult to develop pediatric cancer drugs.

Kids v Cancer is also asking Congress to reform the Pediatric Research Equity Act (PREA) and the Best Pharmaceuticals for Children Act (BPCA). PREA requires drug companies developing drugs that children might use to undertake clinical research of the drugs for children too. However, children with cancer do not benefit from PREA due to loopholes. If we think PREA works for other children's illnesses, we must extend PREA to the most deadly children's disease, pediatric cancer.

Moreover, the pediatric cancer community has a unique opportunity in the 900 drug development pipeline for adult cancers. The FDA only approves about 10-15 of those drugs a

year and there are diamonds in the rough among the drugs that are abandoned. We can create new supplemental BPCA incentives for companies to identify abandoned therapies that could be promising for kids with cancer and repurpose them for kids.

Kids v Cancer is working with Members of Congress on a bill to protect kids with cancer by granting them access to the most promising drugs, which are sometimes investigational drugs and sometimes abandoned adult drugs.

The Creating Hope Act was passed as a result of the joint effort of so many of us. We all changed the world by helping make very sick kids healthier. Thank you.

Now let's get to work to reauthorize the Creating Hope Act and reform PREA and BPCA to better protect kids with cancer.

Warmest regards,

A handwritten signature in blue ink that reads "Nancy". The signature is written in a cursive style with a long, sweeping tail on the letter "y".

Nancy Goodman