

KIDS **V** CANCER

Changing the landscape of pediatric research

December 7, 2016

Dear Friend:

Thank you for your support of Kids v Cancer. As a result, we have successfully championed the Creating Hope Act pediatric PRV, creating almost \$1 billion in R&D incentives for drugs specifically for kids with cancer and other life-threatening illnesses.

On average, each dollar contribution Kids v Cancer has received has resulted in \$600,000 in Creating Hope Act R&D incentives for drugs for kids.

And, Kids v Cancer succeeded in doing this at a time when getting bills through Congress has been especially challenging.

Now, we are working to implement three new initiatives to ensure that drugs developed for adults with cancer are also available to kids with cancer.

This is a special year for Michael and me. Jacob would have been 18. He would have left us and started as a college freshman, somewhere exciting. Michael and I were grateful to have attended the high school graduation of Jacob's friends. Ben, 14, continues to play in his rock band and wrestle. He misses having a big brother, especially when he sees his friends hanging at home with their older siblings. Sarah, 6, has a birthmark on her arm that she likes to say is a heart from Jacob. She recently lobbied Congressional staffers for Kids v Cancer. Her message: "My name is Sarah. I am six. My brother, Jacob, had cancer." She brought the House down.



When Jacob was ill, I was disheartened to learn that although we live in a time of leaps forward in cancer research, children with cancer are largely forgotten. With your support, we have made great strides in identifying problems and crafting solutions so that children with cancer benefit from scientific advances just as adults do.

Problem: There were almost no drugs developed specifically for children with cancer

Solution: The Creating Hope Act

In 2012, Kids v Cancer championed the Creating Hope Act, which Congress passed into law and reauthorized this year.

Under the Creating Hope Act, companies that develop drugs for kids with cancer and other life-threatening diseases receive a voucher from the FDA. With this voucher comes rights to faster FDA review on any future drug. And the voucher is transferable. *Since 2012, the Creating Hope Act has created almost \$1 billion in R&D incentives for drug development for kids with cancer and other life-threatening illnesses.*

We succeeded in an environment in which Congressional action has been especially challenging – only around 2% of all bills introduced are passed into law. *Every dollar you have donated to Kids v Cancer has resulted in about \$600,000 in Creating Hope Act R&D incentives for pediatric rare disease drug development.*

Problem: Of the 900 drugs in the adult cancer pipeline, only a handful are available to children

Solutions to be executed in 2017:

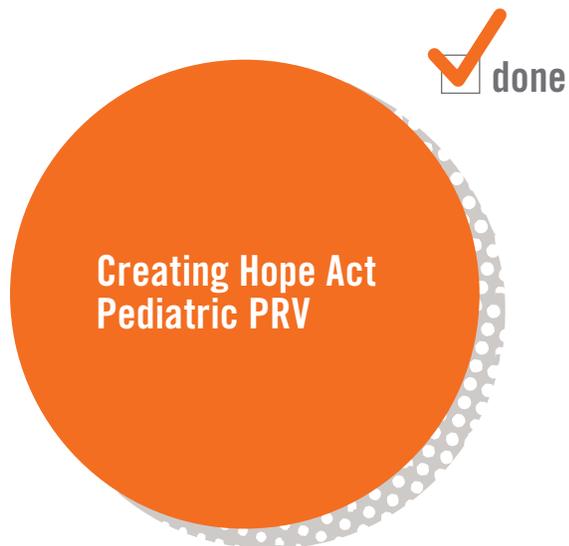
RACE for Children Act

KVC Compassionate Use Navigator

Expanding Eligibility for Trials

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KVC initiatives for cancer drugs that benefit children, but not adults

Our next goal is to create ways for companies developing drugs for adults with cancer to also share the drugs with children. We have three initiatives:

THE RACE FOR CHILDREN ACT provides that companies developing cancer drugs for adults also undertake certain pediatric studies when the molecular targets of the drugs are relevant in pediatric populations.

THE KIDS V CANCER COMPASSIONATE USE NAVIGATOR facilitates physician's efforts to get their kids promising unapproved cancer drugs for treatment.

THE EXPANDING ELIGIBILITY REQUIREMENTS FOR TRIALS addresses the arbitrary manner in which adult trials of promising novel drugs exclude kids under 18 years of age even when there is no scientific rationale for exclusion.



KVC initiatives for cancer drugs that benefit adults and children

Why we need your help in 2017

In 2017, Kids v Cancer plans to roll out these three initiatives to ensure that kids with cancer can have access to drugs developed for adults.

We are asking Congress to pass RACE for Children Act in June 2017.

We are marketing our Compassionate Use Navigator so that children have better access to unapproved drugs.

We are supporting FDA and NIH so that cancer trials include children unless there is a scientific or ethical rationale not to.

With the roll out of these programs, there will be a *dramatic increase* in novel and promising adult cancer drugs for our kids. Sick kids will be healthier. Kids with cancer will live longer. Kids with cancer may be cured.

We have a fantastic Kids v Cancer team. Our team includes Elena Gerasimov, our programs director; Leika Uzcategui, our business manager; and Jenn Flynn, our outreach coordinator. In addition, we have a bank of pro bono strategists, lobbyists, communications experts, lawyers, and writers. We work with scores of advocates and volunteers and youth board members.

Thank you for all you've provided us — your love, your counsel, your encouragement, your participation and your financial support have all been invaluable.

Warmest regards,

Nancy Goodman and the
Kids v Cancer Team

The Kids v Cancer Agenda, 2009 - 2016

Problem #1: There were no drugs developed expressly for kids with cancer

When Jacob was in treatment for medulloblastoma, there were no drugs that had been developed for his disease. Moreover, in the 25 years prior to Jacob's diagnosis, the FDA had only initially approved one cancer drug for children.

To address this problem, we wrote and championed the Creating Hope Act to create an industry-funded incentive.

Solution: Kids v Cancer's Creating Hope Act

The Creating Hope Act works by establishing a voucher system under which companies that develop drugs for kids with life-threatening illnesses receive a voucher from the FDA. With that voucher, the companies can receive faster FDA review on a future drug, perhaps a blockbuster drug. And, the vouchers are transferable and have been sold for hundreds of millions of dollars. The last voucher sold for \$350 million, constituting a strong incentive for pediatric cancer drug development.

Since 2012, the Creating Hope Act has created almost \$1 billion in R&D incentives for pediatric drug development.

What has been so exciting is to see the proliferation of academic researchers and small biotechs use the incentives of the Creating Hope Act to develop pediatric cancer drugs. The FDA has now published a Draft Guidance for Industry on the Creating Hope Act. The Creating Hope Act has been a subject of its own conference and has been covered extensively in the U.S. press.

The Creating Hope Act was passed as a pilot program and would have expired in March, 2016. After two short-term extensions, Congress extended the Creating Hope Act again to September 20, 2020. We thank Representatives Michael McCaul, G.K. Butterfield, Chris Van Hollen and Senators Bob Casey and Johnny Isakson for their dedication to helping children with cancer. We plan to keep working on achieving permanent reauthorization of the Creating Hope Act.

Problem #2: Drugs developed for adult cancers are not also developed for children with cancer

The Creating Hope Act addresses the problem of a lack of incentives for drugs developed expressly for children. However, often there are drugs developed for adults that could benefit children *as well*.

There are 900 drugs in the adult cancer pipeline but few of them are studied for children with cancer. Those drugs that are studied in children are often studied years and even decades after they are developed for adults.

When Jacob's options had run out, it was heartbreaking to read about the many drugs developed for adult cancers that were not being developed for pediatric cancers. We tried so many drugs off label, with scant information about dosage, toxicity, or efficacy.

Solution: Kids v Cancer's RACE for Children Act (S. 3239, H.R. 5858)

In July 2016, Congress introduced Kids v Cancer's Research to Accelerate Cures and Equity for Children Act (RACE for Children Act). The RACE for Children Act provides that companies developing drugs for

adults with cancer also undertake certain pediatric studies. This is not a new obligation. The RACE modifies an existing law, the Pediatric Research Equity Act (PREA) that requires companies developing drugs for adults to undertake studies of kids.

Until now PREA has not applied to kids with cancer because kids often have cancers in different parts of their bodies than do adults. However, cancer drug development is now guided by molecular target. The RACE Act updates PREA so that the PREA pediatric studies will be undertaken by molecular target as well.

The RACE for Children Act has been endorsed by *Nature*, by dozens of hospitals, and by over 150 pediatric cancer advocacy groups.

Kid v Cancer would like to thank Senators Michael Bennet, Marco Rubio, Chairman Lamar Alexander and Ranking Member Patty Murray for their leadership on this effort. In addition, Kids v Cancer once again thanks Representatives Michael McCaul, G.K. Butterfield, and Chris Van Hollen for their continued dedication to finding better cures for children with cancer.

Join us in asking Congress to pass RACE for Children Act in 2017!

Problem #3: Terminally ill kids with cancer can't get access to the most promising, unapproved drugs

The RACE for Children Act creates a mandate for companies to undertake pediatric *studies* to learn the best dosing and scheduling of drugs. However, often there is information about possible efficacy of unapproved drugs for adults. Terminal children with no possible treatment options may just want a chance at access to the drugs.

As Jacob grew sicker, we applied to eight companies for compassionate use access to their drugs. It was a confusing and time consuming process, and none of the companies agreed to share their drugs with Jacob.

Solution: Kids v Cancer Compassionate Use Navigator

Kids v Cancer is proud to announce that in April 2016, we launched the Kids v Cancer Compassionate Use Navigator. The Compassionate Use Navigator provides detailed guidance, forms, and templates for physicians and families seeking access to unapproved drugs, including how to request the authorization of the manufacturer, FDA and a hospital's IRB. The Navigator is also building a first-ever registry of compassionate use requests, tracking the success of applications and clinical outcomes, and seeks to build partnerships with clinical teams to help them in the application process.

We have attended numerous medical conferences to present our Compassionate Use Navigator, and conducted a study of pediatric oncologists' experiences with compassionate use. This work helped us identify the challenges and needs for reform of the expanded access program.

Problem #4: Adult cancer trials of the most promising, novel drugs exclude kids by arbitrarily setting 18 as the minimum age of eligibility with no scientific rationale

There are so many more drugs under development for adults than for children. Often there is no scientific or ethical rationale for excluding children and teens from adult cancer trials. It is age discrimination with harmful results.

Solution: Kids v Cancer is supporting FDA and NCI efforts to expand eligibility requirements of adult cancer trials.

Kids v Cancer thanks the FDA and NIH for their commitment to addressing this issue. Kids v Cancer looks forward to supporting the efforts of the FDA and NIH in establishing and, when possible, applying and enforcing eligibility requirements for adult cancer trials that do not result in exclusion and age discrimination for children.

Problem #5: Molecular targets of pediatric cancers are not all known

Drug development can only be effective if the molecular markers of a cancer are fully understood. And, to undertake such studies, tumor tissue is critical.

Solution: Kids v Cancer's autopsy tissue donation program

Kids v Cancer has been working with families of kids with terminal brain cancers to help facilitate autopsy tissue donations to researchers. Our efforts have indirectly resulted in several *Nature* publications that we hope will result in new treatments for kids.

Kids v Cancer Recognition

We have been honored to have been recognized for our work.

- *NATURE* endorsed the RACE for Children Act
- Congress reauthorized the Creating Hope Act
- RACE for Children Act has been introduced
- Nancy testified before Congress and spoke at The Harvard/MIT Broad Institute, New York Academy of Science, Cornell Entrepreneur Summit, FDA and NIH
- Elena presented the Compassionate Use Navigator at AACR and ASPHO
- The Peter Drucker Institute named Kids v Cancer #1 Innovative Nonprofit
- Fast Company named Kids v Cancer #4 in the top 10 most innovative nonprofits category

Kids v Cancer Youth Lobby Days

One joyful and effective innovation we developed in 2016 was to amplify our voice on Capitol Hill by only using Youth Advocates through our Youth Lobby Day program. For me, it's a special moment to work with Jacob's friends, his brother, Ben, and his little sister, Sarah. If you have a young adult who would be interested in lobbying with us, please let us know. We would love to have them.