

**2<sup>nd</sup> Annual Childhood Cancer Summit**  
Hosted by the House Childhood Cancer Caucus  
September 23, 2011

*Prepared remarks by*  
Nancy Goodman  
Executive Director  
Kids V Cancer

Congressman McCaul, Congressman Van Hollen, Members of the Childhood Cancer Caucus: thank you for inviting me to the Congressional Childhood Cancer Caucus' Second Annual Childhood Cancer Summit to talk about the need for the development of new drugs for pediatric cancer. And specifically, thank you for inviting me to talk about legislation we are championing, The Creating Hope Act, which I'm proud to announce Congressman McCaul and Congressman Butterfield introduced today in the House of Representatives.

#### JACOB'S STORY

I am here today because my son, Jacob, died two and a half years ago of a pediatric brain cancer. Jacob was a beautiful 8 year old boy with red hair and a brilliant, kind smile when he was diagnosed. Overnight, he went from being loud and precocious kid who loved to play any sport with a ball, to a boy in a wheelchair who at times couldn't talk, who had serious cognitive impairments and who couldn't eat or control his basic bodily functions. He spent 9 of his remaining 23 months inpatient in hospitals and much of the rest in and out of clinic, but he carried his burden with a smile and good humor. He was so brave. A trip to the M&M store in Times Square on the way home from a clinic visit was always more interesting to Jacob than dwelling on how much he hurt, or when he'd have to next go back for more treatment.

Today, if he hadn't been sick, he would have been 13. He would have been bar mitzvahed this year. I think he would have been tall and skinny, with a cracking voice. Perhaps he would have complained about homework. I know he would have spent hours a day talking to his younger brother, Ben. He would have loved to have known his baby sister, Sarah.

If there had been effective drugs to treat Jacob, I would have had the chance to watch him grow into a healthy young man instead of speculating like this. But two weeks after Jacob's diagnosis, Jacob's doctors knew the drugs they intended to give him were unlikely to be effective. Yet they continued to use them because there were no alternatives. No new drugs have been introduced to treat Jacob's form of cancer in decades.

## FACTS OF PEDIATRIC CANCER

Pediatric cancer is thought of as a terrible, but obscure problem.

Let me say this: pediatric cancer is not obscure. Thousands of children die of cancer every year, missing their whole lives when they die. And, the lucky ones – the hundreds of thousands of survivors -- live with a raft of impairments and often life threatening conditions. Measured in years of life lost and QALYS pediatric cancer has an impact that rivals breast cancer.

And the death of even one child is something that has a great impact on families and communities. There's something particularly unjust about a kid dying of a disease that should be treatable. It just sticks with you.

I want to share with you a few of the things I learned about pediatric cancer through the experience of taking care of Jacob, talking to dozens of parents with sick children and consulting with literally scores of research institutions in search of better treatments.

What I learned is the following:

First, pediatric cancer is different from adult cancer in a number of ways

- Jacob's form of brain cancer occurs in adults only exceedingly rarely
- And evidence strongly suggests that cancers that occur in both children and adults have different molecular biologies.
- Whereas adult cancers are often environmental, pediatric cancers tend to be developmental;
- Treatments for cancers in children and adults are different.

So it is not always the case that a drug developed for an adult cancer is effective in children. Moreover, there are many instances in which a cancer may benefit a child, but not an adult. These drugs never get developed.

Second, there have been no new drugs developed for pediatric cancer in many years. In fact, in the past 20 years, the FDA has initially approved only one drug for any childhood cancer.

Third, unless we do something, it is unlikely that many new drugs will be developed for pediatric cancers in the future, either. Because the childhood cancer drug market is so small, there's no private drug company funding to develop new drugs.

Fourth, for a number of structural reasons I can go into at another time, at 4% of the National Cancer Institute budget, pediatric cancer research is underfunded by the government as well.

Now let me be clear: There has been progress. But statistics can be misleading. The adult cancer community measures progress in terms of five-year survival rates, but improvements in five year survival rates don't really matter when your child dies in his 20's of a secondary cancer.

And almost none the long term survivors achieve a "cure". The word cure implies a return to health. To increase survival levels, our kids now get doses of highly toxic chemotherapies and radiation that leave them scarred for life, often requiring ongoing, serious medical attention. So, perhaps more survive, but they survive much sicker – not because of the cancer, but because of the treatments themselves. Had Jacob survived, the treatments he received would have rendered never able to live a normal and independent adult life.

Since Jacob's death, my focus has been to try and eliminate the obstacles that stand in the way of new drugs being developed to treat pediatric cancer and other pediatric rare diseases.

Through Kids v Cancer, we are launching a tissue bank to make rare tissue available to researchers to help them solve the mysteries of pediatric brain cancer. We're beginning a dialogue with the FDA about whether there are ways that agency can do more to promote pediatric cancer drug development. And, with Congress, we're focused on how to create market incentives for pediatric drug development.

#### CREATING HOPE ACT

We live in the country with the world's leading researchers and scientists, and yet almost none of that talent is being directed toward drugs for our children. The problem is that the size of the drug markets for pediatric cancer drugs is too small for drug companies to enter. We have to find ways to incentivize drug companies to enter these markets. There have been a number of legislative efforts to promote new drug development, but frankly, they have not been effective for pediatric cancer drugs. Extending patent protection or market exclusivity for drugs expressly designed for pediatric cancer does not provide a sufficient incentive because the markets are too small for that patent protection to be valuable. The incentives must occur outside the market of the pediatric cancer drug.

The Creating Hope Act builds on the neglected tropical disease priority review voucher by improving it and expanding it to include pediatric cancer and other pediatric rare diseases. In designing the Creating Hope Act, we've tried to take into account the fact that this is not the best time to propose an increased appropriation or a costly program. I

want to also note that the Creating Hope Act also closes certain loopholes that could create a windfall for pharmaceutical companies.

Under the Creating Hope Act, if a pharmaceutical company gets all the way to FDA approval for a drug that addresses a pediatric cancer or other pediatric rare disease, a voucher would be awarded. The voucher would come with rights to a priority review for any drug that company produces. This would result in that other drug being introduced to market more quickly once approved.

The Creating Hope Act requires no appropriation, but it requires real results for a real benefit.

The Creating Hope Act is broadly supported not only by parents like me and advocacy groups of the pediatric cancer and rare disease community, but medical researchers and drug companies. It has bipartisan support in the Senate and now, I'm proud to say, in the House of Representatives. This is a reasonable approach to addressing the absence of drugs developed expressly for pediatric cancer and other pediatric rare diseases.

I urge all Members of Congress to support this bill and shepherd it into law. If you've known a child who died – particularly of a condition that should be treatable – you know what a profound impact it has – including on the family and on the community. In this country, we should not tolerate the death of thousands of children each year and the condemnation of tens of thousands more to lives of significant impairment. We cannot allow this without making an effort to solve this problem. We can do better. We should try. The Creating Hope Act is one step in that direction.

Thank you.