

“The Impact of Pediatric Cancer on children and families:
why we need to Change the Landscape of Pediatric Cancer Research”

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I would like to thank Congressman Michael McCaul, Congressman Joe Sestak, and the entire

Pediatric Cancer Caucus for allowing me to speak today on behalf of children diagnosed with cancer.

I am the Founder and Executive Director of Kids v Cancer but I speak to you, first and foremost, as a parent who has recently lost a child to brain cancer. My son, Jacob, was your typical, wonderful eight-year old boy when he was first diagnosed: a sweet kid, a good brother, an able student, an enthusiastic athlete, and a passionate rock-and-roll keyboard player and vocalist. After some unexplained morning headaches and nausea, we were told that Jacob had medulloblastoma, a form of pediatric brain cancer. Jacob endured several surgeries, six weeks of daily radiation and many difficult chemotherapy protocols. He suffered severe neurological and cognitive impairments, including an inability to speak or, for a time, move. He was wheelchair bound, he lost gross and fine motor skills, he experienced significant memory deficits. Jacob had unmanaged pain, anorexia, nausea, baldness and multiple infections. He was hospitalized for months at a time. And still, unbelievably, he never complained and just focused on his friends, his family, and attending school as much as he could. Jacob died a year and a half ago at age 10.

During our hard-fought efforts to save our son, we came to realize that Jacob and children with pediatric cancer are at a serious disadvantage when it comes to research for treatments and a cure.

In Jacob's honor and for every other child with pediatric cancer as well as those who have not yet been diagnosed, I started Kids v Cancer, a nonprofit dedicated to changing the landscape of pediatric cancer research by providing support to the pediatric cancer research community.

At the heart our efforts are the very sobering statistics on the allocation of resources to combat pediatric cancer.

The FDA has had an initial approval of only one pediatric cancer drug in the past 20 years. The National Cancer Institute spends less than 4% of its budget on research for pediatric cancers.

And the incidence of invasive pediatric cancers has increased by 29% over the past 20 years.

If you look at these statistics, you can see there is clearly a crisis in the system that your Caucus should be commended for highlighting today.

Our goal is simple: to bring pediatric cancer researchers the resources they need to find better treatments for childhood cancers. Our efforts are focused in three areas: access to new drugs, tumor tissue and funding.

[The Cost of Pediatric Cancers](#)

All cancers are terrible and impose tremendous costs on the diagnosed. But childhood cancers are in a class all their own. The loss of a child to cancer is among the worst tragedies a family and a community can endure, the pain magnified by the knowledge that such an early death represents the loss of an entire lifetime's hopes, dreams, and experiences.

The impact of pediatric cancers on families and our society is devastating. Each year about 13,500 children are diagnosed with cancer in the United States, and for 25% of them, that diagnosis is fatal. Though some childhood cancers, such as leukemia, have seen significant improvements in survival rates, others have witnessed no improvements in decades. Many pediatric cancers, including Jacob's disseminated medulloblastoma are terminal upon progression or recurrence. Others are terminal at diagnosis.

More children die of cancer every year than the terrible terrorist attacks on our country just ten years ago. Cancer kills more children than AIDs, asthma, diabetes, cystic fibrosis and congenital anomalies combined. When a child dies of cancer, that child loses almost 70 years of life. Jacob was 10 when he died. He missed dating, graduating high school, going to college, finding a first job, getting married, having children. While we have made significant progress in addressing certain cancers, these numbers are still too high for any child.

For those children fortunate to survive their diagnosis, the vast majority face chronic illnesses and life-long impairments, including secondary cancers, impaired cognitive and social abilities, heart damage, lung damage, infertility and alternations in growth and development. In many cases, the toxic treatments themselves are the cause of life-long challenges. Childhood cancer treatments tend to be at relatively higher doses than adult treatments, and with those higher doses come more serious long-term effects. To put this in perspective, children with Jacob's diagnosis who receive radiation to their brain before age 8 may have a difficult time ever counting money and doing multiplication and division. Children who receive cranial radiation before age 5 may never learn to read or tell time. And babies who receive cranial radiation may never learn to talk. Had Jacob survived, he probably would not have been able to stay in his mainstream school, go to college, or live independently.

The image of a bald, frail child in a hospital gown on a fundraising brochure strikes a blow deep into the soul of a community that values and cares for its children, and for decades Americans have been opening their hearts—and their wallets—to support cancer research. But what people don't know is how few dollars of those dollars go to fight pediatric cancers and how much the pediatric cancer research community needs funds, new drugs and tissue to make real progress.

Much more needs to be done. It is time to shed light on the ways that we, as a country, are failing our children. Policy makers, regulators, health-care professionals, and parents must be made aware

of the crisis that faces every new generation.

II. Addressing the Impediments to Childhood Cancer Research

Pediatric cancer researchers are a gifted and dedicated group of people who work under resource-scarce conditions to try to understand the causes and mechanisms and, therefore, better treatments and cures for childhood cancers. What stands in the way is access to new drugs, available tissue for research and adequate funding. Let me touch briefly on these three areas.

New Drugs

Within weeks of Jacob's diagnosis, it became clear that his tumors were unresponsive to the standard combination of radiation and chemotherapy. The protocol our medical team was planning to give Jacob was unlikely to work, yet they proceeded nonetheless because there were no other reasonable alternatives. Over the past thirty years, treatment for medulloblastoma has not materially changed because federal funding for new drugs for medulloblastoma is near zero, and because no pharmaceutical company can afford to engage in research and development for new drugs for children like Jacob without significant governmental assistance because the market is too small.

What drug innovations there are for childhood cancers come from hand-me-down adult drugs that may coincidentally have efficacy for children too. This is a poor strategy. Childhood cancers tend to have different root causes than adult cancers and require unique drugs. Yet, in the past 20 years, the FDA has initially approved only one new drug, clofarabine, for a pediatric cancer.

We need to create market incentives for biotech and pharmaceutical companies to invest in the development of pediatric cancer drugs. In that regard, Kids v Cancer applauds Senators Sam Brownback and Sherrod Brown, a Republican from Kansas and a Democratic from Ohio, who introduced the Creating Hope Act of 2010, a bill that would provide incentives for biotechnology and pharmaceutical companies to develop drugs for pediatric cancers and other pediatric rare diseases through a priority review voucher program. Under this program, a company or institution that develops a drug for a pediatric cancer and receives FDA approval for that drug also receives a voucher. That voucher comes with rights to expedited FDA approval for any other drug – most likely a blockbuster drug– which results in the blockbuster drug getting to market many months earlier. The voucher would be fully transferable and is estimated to be worth in the hundreds of millions of dollars. For a more detailed review of the Creating Hope Act, please refer to the Kids v Cancer website at www.kidsvcancer.org.

The Creating Hope Act of 2010 builds upon the priority review voucher program established by the FDA Amendments Act of 2007. Chiefly, this legislation would expand priority review

voucher eligibility for neglected tropical diseases to also include rare pediatric diseases. The Creating Hope Act would strengthen this legislation via a series of technical improvements intended to support the value of the vouchers and ensure that vouchers are only created when bona fide new drugs are created. Among the modifications introduced in the Creating Hope Act are: the closure of a loophole that authorizes vouchers to be issued for tropical disease drugs that were already approved and marketed abroad, the transferability of the voucher, a better definition of what constitutes a new drug, provisions to promote the marketing of the underlying drug, FDA notification requirements that do not undermine the value of the voucher and an upfront priority review voucher designation process.

Kids v Cancer hopes the Pediatric Cancer Caucus and the entire pediatric cancer community will join us in advocating for passage of this important legislation.

Tissue

In addition to access to new drugs, pediatric cancer researchers also need access to tissue to better study the biologies and test potential treatments for childhood cancers. When Jacob died, we arranged for his tissue to be sent to four different researchers whom we had consulted during Jacob's treatment. Of the four researchers, two achieved significant breakthroughs using Jacob's tissue. One performed the first genetic analysis of primary versus metastatic tissue on a medulloblastoma and determined, very importantly, that the metastases are fundamentally different than the primary tumor. I understand that an article will be forthcoming in part based on that investigation. Another researcher harvested viable tumor cells from the postmortem tumor tissues and by injecting these tumor cells into the brains of immunodeficient mice, he built the first ever xenograft postmortem brain tumor tissue mouse model for any diagnosis of brain cancer, pediatric or adult. That we had two major breakthroughs out of four researchers is not remarkable; it only underscores how much good science is waiting to be done if pediatric researchers can get their hands on the necessary materials.

Toward this end, Kids v Cancer is launching a parent-led pediatric postmortem brain tumor tissue donation program. Through our website, we will provide outreach and support to parents of children with terminal brain cancer, letting them know that tissue donation is an option and providing them information about how it works. For physicians, we will be offering counseling on how to approach parents about tissue donation, as well as logistical information about how to execute on a request. We will also provide information about researchers seeking postmortem tissue and their projects.

Access to Federal Funding

The National Cancer Institute spends over \$4.8 billion in health care, but spends only \$190 million, under 4% of its budget, on research for all pediatric cancers. NCI funding levels are particularly important for pediatric research because, unlike adult cancers, where because of the size of the potential market, 60% of the research funding comes from private industry, private funding for pediatric cancer research is negligible.

However, while childhood cancers are low incidence cancers, these funding levels are not proportionate to the possible impact of childhood cancer research when the age at diagnosis or death, the years of lives lost, the decreased quality of life for childhood cancer survivors, the 30 year survival rates or the overall value our society places on a healthy childhood are taken into account.

We as a community must consider numerous issues to understand how to achieve the most appropriate quality and quantity of federal funding of pediatric cancer research, including by removing any structural impediments to an optimal allocation of resources. For example, does the NCI and NIH peer review process undervalue important and unique aspects of childhood cancer research such as years of life lost with the death of each child, poor quality of life for childhood cancer survivors, and the value our society places on protecting children? Do NIH and NCI peer review scoring guidelines that narrowly define public health impact disfavor important knowledge that can be gained from childhood cancer research and instead focuses on next-step innovations in the best-studied cancers affecting adult patients?

Furthermore, does the NCI's process for establishing directed grants adequately address the opportunities and needs of pediatric cancer research?

Should pediatric cancer research be a higher priority for other government funding sources?

In the coming months, Kids v Cancer will be exploring these and other funding questions.

Conclusion

Starting in the 1960's, substantial effort was dedicated to addressing pediatric leukemia. And, since then, survival rates have climbed dramatically. When adequate resources are put to the task, we can find better treatments and cures. We are in a time of unprecedented scientific breakthrough.

Through microarray analysis, we are gaining a better understanding of the genetic mutations, activated pathways and the mechanisms by which cancers develop. We are on the verge of being able to design personalized strategies to treat specific patients. Through structural biology, we can design new drugs to match newly discovered genetic mutations, and immunotherapy is providing a new paradigm for thinking about cancer treatment.

Our hope is that children with cancer will fully benefit from this exciting scientific time. Kids v Cancer will continue working to promote passage of the Creating Hope Act of 2010 to encourage new drug development. We will work to expand the availability of tissue to cutting edge researchers. And we will work to increase access to funding to ensure that every worthy idea in pediatric cancer research is fully explored.

We appreciate the interest, commitment and support of the Pediatric Cancer Caucus and look forward to working with you toward these objectives so that one day, children like Jacob will be able to live out full, healthy and productive lives.

Thank you.