



PAC2 Report on the Pediatric Cancer Caucus – September 16, 2010

PAC2 was honored to be invited and attend the briefing to Congress presented by the Pediatric Cancer Caucus. The event was held in the Rayburn House Office Building, directly across from the Capitol. It started early and finished late.

The briefing was presented by an incredible mix of men and women with the knowledge, ideas, dedication and passion to cure childhood cancer. So excuse the lengthy nature of this report, which will include both facts and my thoughts, but I'm compelled to convey as much as possible. I wish I could take shorthand because so much was said. Please excuse any errors and note that most are not direct quotes (unless noted with quotations), but my recollection and scribbling and are only intended to convey the message - which was one of hope....

As I made my way home, I just wished that each and every member of the childhood cancer community could have been there.

The briefing was hosted by Congressmen [Joe Sestak](#) (PA) and [Michael McCaul](#) (TX) and intended to demonstrate to Congress the urgent need for increased federal support to cure kids cancer. CureSearch and Children's Cause for Cancer Advocacy also hosted a pre-briefing breakfast. The panelists included:

- [Dr. Peter Adamson](#) - Chair, Children's Oncology Group
- [Dr. Eugenie Kleinerman](#) - Head, Division of Pediatrics, [M. D. Anderson Cancer Center](#)
- [Dr. Susan Blaney](#) - Deputy Director, [Texas Children's Cancer Center](#)
- [Dr. William Evans](#) - Director and CEO, [St. Jude Children's Research Hospital](#)
- [Dr. Melissa Hudson](#) - Director, Cancer Survivorship Division, [St. Jude Children's Hospital](#)
- [Dr. Kevin Oeffinger](#) - Director, Adult Long-Term Follow-Up Program, [Memorial Sloan-Kettering Cancer Center](#)
- [Congresswoman Deborah Pryce](#) - Founder of [Hope Street Kids](#) and the [Caroline Walker Pryce Conquer Childhood Cancer Act](#)
- [Dr. Susan Weiner](#) - Founder, [Children's Cause for Cancer Advocacy](#)
- [Nancy Goodman](#) - Founder, [Kids V Cancer](#)

The room was full to capacity, with many standing in the back. ABC News was there, and further info will follow. A partial list of other attendees includes:

- [Congressman Jim Moran \(VA\)](#)
- [Congresswoman Jackie Speier \(CA\)](#)
- [John Lehr](#) - President and CEO, [CureSearch for Children's Cancer](#)



- [Cynthia Duncan](#) - Director of Advocacy, [CureSearch for Children's Cancer](#)
- [Sally Charney](#) - Director of Public Education, [CureSearch for Children's Cancer](#)
- [Lori Salley](#) - Executive Director, [Children's Cause for Cancer Advocacy](#)
- [Ruth Hoffman](#) - Executive Director, [American Childhood Cancer Organization](#)
- [Joe McDonough](#) - Founder, [Andrew McDonough B+ Foundation](#)
- [David Plotkin](#) - Founder, [Max Cure Foundation](#)
- [Vicki and Peter Brown](#) - Founders, [Mattie Miracle Cancer Foundation](#)
- [Donna and Tim Culliver](#) - Founders, [Adam's Angels Ministry](#)
- Matthew Alsante - Executive Director, [Sarcoma Foundation of America](#)
- Representatives from [Alex's Lemonade Stand Foundation](#), [St. Baldrick's Foundation](#) and others

I think it is important to note that while pharmaceutical companies were invited to attend unfortunately there were no representatives from the folks that bring you Cialis for that issue, Nexium so we can all eat at McDonald's forever and of course, the cure for restless leg syndrome. I've always wanted to ask if they had suggested language to relate to kids why profitability concerns preclude drug research for kids cancer; but maybe I'm just [ODD](#)? [that's it on cynicism (for this post); I'm feeling too good]

The opening remarks by **Rep. McCaul** were brief but important. Today represented the largest collection of childhood cancer experts ever assembled before Congress. McCaul related the story of his introduction to childhood cancer through meeting with Tim and Donna Culliver of Adam's Angel's Ministry. And hailed The Carolyn Pryce Conquer Childhood Cancer Act as the most non-partisan effort ever.

Dr. William Evans of St. Jude then spoke. Like each speaker, his remarks were hopeful and forceful. Some key points from his presentation:

- WE, the entire community, are pleased the cure rate is 75%, but we must do better
- New drugs for adults do not cure childhood cancer
- Genomics and targeted therapies are the wave of the future, which we **MUST** make NOW
- The \$60 million [St. Jude/Washington University Genomics Project](#) will last at least 3 years and decode more than 600 kids genes
- This project will provide a platform for new discoveries leading to better treatments
- The missing link is resources; the Caroline Pryce Walker Conquer Childhood Cancer Act and the Creating Hope Act of 2010 are essential to moving forward

Dr. Evans concluded:

"This is the most exciting time in my 30 year career!"



Congressman Jim Moran was up next. Sixteen years ago his daughter Dorothy felt like she had the flu. After 7 trips to the doctor and hospital, he finally enlisted the aid of a friend at NIH. Finally, she was diagnosed with a brain tumor, with a 10% chance of survival. But finally, after **"cutting, poisoning and burning"**, she survived. Today, she is alive, with long-term complications, but alive. He was very moving. Anyone in VA needs to fully support Mr. Moran.

Former Congresswoman Deborah Pryce then encouraged the community to **"keep telling your stories"**, and keep the pressure on! That's what we do, right? She called for more innovative thinking to solve this problem without simply throwing money at it. That serves as the lead-in to later discussions for incentivizing big drug companies to participate. She encouraged Sestak and McCaul to make this an annual event before giving way to the doctors. And to ensure that drug company reps are included next year.

Peter Adamson is, of course, the Chair of the Children's Oncology Group (COG). And a wonderful speaker. COG is the collection of 230 hospitals in the US and internationally that collaborate on childhood cancer research. He has worked for 25 years in experimental treatments. Many times, as the options grow short, he is asked "If it were your child, what would you do?" As Joe and I sat there, nodding our heads, Adamson continued "What I say is that I can't tell you, even after 25 years of doing this, because I just can't even imagine sitting on that side, or what you're feeling, it is unimaginable. But I can help guide you." He so wants that to change. Adamson spoke of Jordon, a patient with ALL. She was treated with 11 drugs. About 1/2 of them were developed in the 1950's, another 1/2 in the 1960's, and one was developed in the 1970's. Cure rates may have climbed, but only because doses have increased. But that in turn results in the majority of survivors battling long-term health consequences. Jordan is now 16 years old and has a hip replacement. If I heard correctly, he said that 4 of 5 kids experience severe, life-threatening, or fatal side effects - **during treatment!**

He described COG as the "largest medical/scientific collaboration in history". But what can Congress do to bring pharmaceutical expertise and talent to the table? One example is the [Better Pharmaceuticals for Children Act](#), which is up for renewal. And he expressed his whole-hearted support for the "Creating Hope Act of 2010". Dr. Adamson concluded:

"We are entering an unprecedented time in history. We will soon understand the fundamental drivers of childhood cancer. We will have failed a generation of children unless we do something with it. The most optimistic group out there is pediatric oncology doctors. Let today be the tipping point so that in 5 to 10 years, there are no more unimaginable stories."

Tough act to follow, but **Dr. Susan Blaney from TCH** is no rookie! She spoke about barriers to drug development. There are two basic impediments: 1) practical and 2) scientific. Barriers include:



- The marketplace for development of new drugs is small. Only 1% of cancer cases involve children, it's an orphan disease. That means it's not profitable for the drug companies to develop new treatments.
- Drug companies also have concerns that if 'negative impacts' are observed early in the FDA approval process, it will delay roll-out, and thus profits
- Investigators simply spend too much time completing grant applications instead of doing actual research
- Because pills can't generally be swallowed by young kids, this delays the use of formulations
- Now they are finding that tumors are not really just one type, but can contain 2 or 3 or 4 different types

Dr. Blaney concluded:

"Once the genomic work is completed, we will be able to develop new drugs. We have the tools and technologies; but need funding. Time is of the essence."

Dr. Kleinerman from MD Anderson is no holds [barred](#). A 30-year veteran, she opened with the usual, most drugs were developed 30-40 years ago and the molecular abnormalities in kids are different than adults; different cancers. She talked openly and frankly about her frustrations in obtaining funding. She is recognized as a worldwide expert and sits on some of the grant review bodies. She says that excellent pediatric grant applications are losing out to mediocre adult grants because the review body only has one pediatric perspective and the others deem the adult drug to be more important to the cancer mission generally. Even though the science behind her application is better than the competing proposal. So, what's the fix?

She introduced the concept of a **Childhood Cancer Study Section within NCI for pediatric cancer research study evaluation!** This will incentivize researchers to apply because they will know that the application will be judged by someone in-tune with ped-onc research. This is truly a huge and important idea that can help level the playing field for us! Dr. Kleinerman concluded:

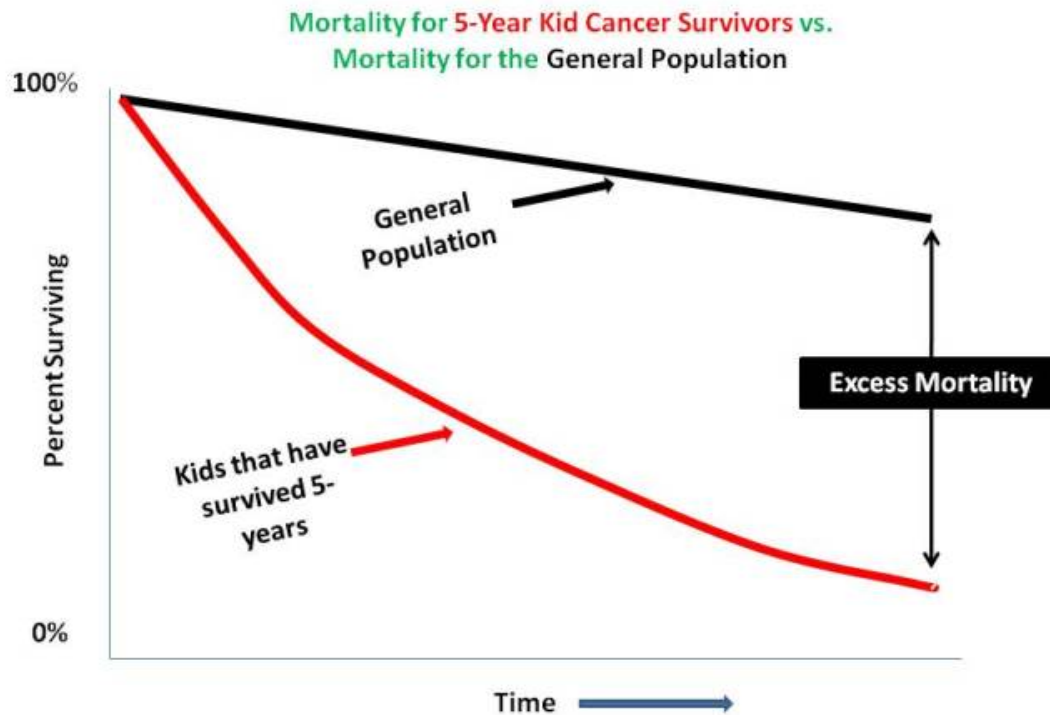
"Curing childhood cancer is the equivalent of curing breast cancer in terms of productive life years saved"

Heard [that](#) somewhere!!

Drs. Hudson and Oeffinger then spoke about survivorship and [long-term effects of treatment](#). One in 570 adults is a childhood cancer survivor - there are currently over 300,000 survivors. They presented further information from the [Childhood Cancer Survivor Study](#) conducted by [30 participating institutions](#) that looked at 14,000 kids and siblings. One eye-catching chart shows the excess mortality. This essentially tracks the number of kids that have survived 5-years, yet still experience an early death resulting from issues associated with either recurrence or complications from treatment. So this is what happens with the 78% of survivors, [excess mortality](#). Ugh. The chart starts at the time when a kid has survived 5-years, and then tracks the outcome as compared to the general populations. The difference is the excess mortality.



(Note: graph created by PAC2 and is only intended to demonstrate the concept, excess mortality. Just like the 22% of kids diagnosed that die, this must also be reduced to 0%. I will track the actual data down and provide asap)



The doctors stated that 73% of survivors had at least one chronic health effect, 42% had very severe or fatal effect, with 10% developing a secondary cancer.

But aren't we doing better now, with 'new' treatments? A case study of a mom and daughter both diagnosed with childhood cancer was presented to take a look at that question. Talk about unimaginable huh? The mom was diagnosed in 1975 with leukemia. My notes on her effects from treatment read 'the list goes on and on'. The daughter was diagnosed in 2002 at the age of 14. Her list of secondary effects may have been a bit shorter, 'only' including: secondary cancer, a bone marrow transplant, infertility, hip replacement, thyroid problems, diabetes, and iron overload. So, while we are able to save more kids; the long-term effects of the cure/"cutting, poisoning and burning" continue to result in terrible complications, and excess mortality.

So what can be done? They both STRESSED the importance of LONG-TERM FOLLOW UP. If these effects are detected early enough, by a physician who knows what to look for, we CAN improve these results. But while 90% of adults follow-up, only 20% of kids are followed up. As they become young adults they stray from the mix in an attempt to escape the hell they've been



through (my thought).

But there are excellent programs and guidelines in place to help. The [Long-Term Follow-Up Guidelines for Survivors of Childhood, Adolescent, and Young Adult Cancers](#) from COG/CureSearch was referenced, as well as the [Passport for A Cure](#) program in place at TCH/Baylor.

Congresswoman Speier stressed the importance of [H.R. 2109:Childhood Cancer Survivorship Research and Quality of Life Act of 2009](#) she introduced on April 27, 2009 with [Mary Bono Mack](#) (CA). HR 2109 is important bipartisan legislation that will improve and expand the delivery of medical and psychosocial care to survivors of childhood cancer. Both doctors agreed that to minimize these late effects, follow-up and the use of the Guidelines are key.

Congressman Sestak then spoke of his personal story with his daughter, the "only perfect child in the world", yep, him too. I won't relate his personal story and thoughts; I don't want to not get it right, and like Tom Hanks said in Saving Private Ryan when asked about his wife and those rosebushes: "No, no that one I save just for me." But Mr. Sestak is the highest ranking military officer ever voted into Congress, and both commands respect and comes across as a dad at once. He said that he is here today as part of his "payback tour". I hope the tour lasts a long time Mr. Sestak. It was an honor to meet you.

Nancy Goodman of [Kid v Cancer](#) followed. Her son Jacob was diagnosed at 8-years old. He was a brother, an athlete, and a rock-and-roll keyboardist amongst so many other things. He was lost at age 10. Nancy recounted the following sobering statistics:

- The FDA has had an initial approval of only one pediatric cancer drug in the past 20 years
- The NCI 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"

She has been the driving force behind the Creating Hope Act of 2010 to encourage new drug development, expand the availability of tissue to cutting edge research. Kids v Cancer is also working to increase access to funding to ensure that every worthy idea in pediatric cancer research is fully explored.

Her talk stressed the cost of childhood cancer, in terms of pain to the surviving family members and also the "loss of an entire lifetime's hopes, dreams, and experiences." She referenced an "image of a bald, frail child in a hospital gown on a fundraising brochure" yet "what people don't know is how few of dollars that are donated go to fight pediatric cancers". I [agree](#).



Nancy applauded **Senators [Sam Brownback \(KS\)](#) and [Sherrod Brown \(OH\)](#)** who introduced the [Creating Hope Act of 2010](#) to provide incentives for biotech and pharmaceutical companies to invest in the development of new drugs for pediatric cancer and other pediatric rare diseases through a priority review voucher program. When a company develops a drug for pediatric cancer and receives FDA approval they would get a voucher. The voucher provides rights to expedited FDA approval for any other drug which would get that drug to market faster. Vouchers could be worth hundreds of millions of dollars!

Nancy doesn't stop there. When Jacob died, the family arranged for his tissue to be sent to four researchers for study. Two of them achieved significant breakthroughs! One determined that metastases are fundamentally different from the primary tumor, and a second developed the first ever model for diagnosis of brain cancer; pediatric or adult. Toward this end, Kids v Cancer is launching a parent-led pediatric postmortem brain tumor tissue donation program. Lots more to come from Nancy and Kids v Cancer on that!

Well, Nancy doesn't stop there either! Saying that NCI funding levels are particularly important for kids, because unlike adult cancers, where 60% of research funding comes from private industry, private research into kids cancers is negligible. The NCI funding levels are not proportionate to the age of diagnosis of kids, the years lost, the decreased quality of life of survivors, the 30-year survival rates.....or the "overall value society places on healthy kids". Again, I [agree](#).

Nancy concluded:

"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"

Nancy's presentation can be downloaded [here](#). Nancy is 8 months pregnant and we all wish her and her family all the best!

Susan Weiner of Children's Cause for Cancer Advocacy then spoke. I was very excited to hear of another IOM study coming later this month, similar to the [report](#) discussed [here](#). In addition, and just as exciting, was her thoughts on development of a **public-private partnership** to encourage more participation in pediatric cancer research while absorbing some of the risk. Although in its early stages of development, and still needing seed money, the concept was also discussed in the report referenced above.



Congressmen McCaul and Sestak wrapped up this incredibly moving day by saying that we need to keep moving forward to **"solve this crisis."** **The legislative agenda discussed today, including:**

- 1. The Childhood Cancer Survivorship Research and Quality of Life Act;**
- 2. The Creating Hope Act of 2010;**
- 3. Re-authorization of the Best Pharmaceuticals for Children Act; and,**
- 4. Creation of a Childhood Cancer Study Section at the National Cancer Institute**

will be a huge stop in the path to curing childhood cancer.

Simply put; it was incredible. For once, I saw a comprehensive plan of action within the government, with participation from public groups, to address the thing we fight for every day; kids. I came away full of hope, and so inspired by the amazing people that are working to make this happen. They will not be able to implement this in a vacuum, or just because it's right. They will need the help of educated and articulate childhood cancer advocates to support and fight for the steps outlined above with other Senators and Representatives. That's where you come in PAC2.

In the coming weeks and months, they will be seeking your support. PAC2 hopes to continue to serve as a conduit for who, where and when we should, as **Deborah Pryce** put it: "apply the pressure". We will continue to coordinate with these wonderful people that have put forth this plan; share with you what we learn and hopefully advocate together as "one voice united against childhood cancer". I can't think of a more rewarding day.

My thanks to Cynthia Duncan, Lori Salley and Andy Mikulak of [Max's Ring of Fire](#). A special thanks to Joe and Jane, for your support and friendship. And my many thanks to you PAC2, for all you do and for allowing me to represent you, and them.

AJs Dad