HOUSE DEMOCRATIC POLICY COMMITTEE HEARING

Topic: Pediatric Cancer Research Funding

Roberts Center for Pediatric Research – Philadelphia, PA

June 1, 2017

AGENDA

2:00 p.m. Welcome and Opening Remarks

2:10 p.m. Panel One – Researchers:
- John Maris, MD, Pediatric Oncologist and Chair of Pediatric Cancer Dream Team, Children’s Hospital of Philadelphia
- Valerie Brown, MD, PhD, Director of Experimental Therapeutics in Division of Pediatric Hematology/Oncology, Penn State Children’s Hospital

2:40 p.m. Panel Two – The Absolute Destruction of DIPG:
- Bill and Cathy Kohler, Son Ayden passed away from DIPG
- Patrick Dunion, Son Aidan passed away from DIPG

3:00 p.m. Panel Three – Childhood Cancer Warriors:
- Seth Wooldridge, Childhood Cancer Warrior
- Travis Cook, Childhood Cancer Warrior

3:20 p.m. Panel Four – Hershey Medical Center and CHOP Families
- Elizabeth Wooldridge, Mother of Seth
- Jennifer Kratzer, Mother of Collin
- Sara Weinhold, Mother of Noelle
- Beth Caruso Cook, Mother of Travis

4:00 p.m. Panel Six – Survivors and Advocacy
- Carly Nemo Wilfong, Childhood Cancer Survivor and Advocate
- Christopher Winters, President, National Children’s Cancer Consortium

4:20 p.m. Closing Remarks
Importance of Pediatric Cancer Research Funding
And House Bills 1804, 404, 408

Testimony presented by:
John M. Maris, MD
Children’s Hospital of Philadelphia
Co-Director of Pediatric Cancer Dream Team
Giulio D’Angio Chair in Neuroblastoma Research

House Democratic Policy Committee
Pennsylvania House of Representatives

Roberts Center for Pediatric Research
2716 South Street, Philadelphia, PA 19104
Thursday, June 1, 2017, at 2:00 p.m.
Good afternoon. My name is John Maris and I am a Pediatric Oncologist at the Children’s Hospital of Philadelphia.

- First, I want to thank Chairman Mike Sturla and the members of the House Democratic Policy Committee for having this hearing on these important issues and allowing me to represent CHOP and testify.

- I’d also express my sincere gratitude to the visionary leadership of Representative Tom Caltagirone, especially for introducing the package of bills which prompted this hearing. House Bills 1804, 404 and 408 are critically important steps in combatting pediatric cancer here in the Commonwealth.

Cancer is an ominous disease, and it is especially sinister when it affects an innocent child. Before speaking to 5 specific points related to progress, opportunities and challenges related to improving outcomes for children with cancer, I would like to tell you a bit more about myself, as a representative (like Dr. Brown here) of the pediatric oncologist community, to put my words in context and hopefully demonstrate to you how passionate we are about the cause the brings us here today, and to hopefully further instill in you the threats we currently face without action.

I am a physician-scientist. What that means is that my life is a blend of patient care and research. I arrived on this campus in 1983 to do research on childhood cancer, and while I didn’t think then that I would still be here 34 years later, here I am. I have had the privilege of being mentored by internationally renowned pediatric oncologist who also made Pennsylvania their home. They gave me the insights, tools and guidance to build a large research program, make discoveries, translate these discoveries into new therapies for children, and to be a compassionate physician.

- Other than this hearing, today is a typical day—met with incredibly bright students and fellows in my lab early this morning to review experimental plans, met with two patients and their families shortly thereafter to monitor how they are doing on a brand new “personalized” experimental drug we developed for their relapsed cancers, will immediately after this hearing run a conference call for a multi-institutional program I lead to collaboratively develop new immunotherapies for childhood cancer, and then I will drive over the bridge to New Jersey to visit a 12 year-old little girl with neuroblastoma whom I have cared for since she was 4. After front line therapy, relapses and six separate experimental therapies, we have failed her and she will die soon. Indeed, a pet peeve of mine is when I hear or read that a “child lost his/her battle with cancer”—that child lost nothing—it is we as a health care community that lost the battle, and indeed is why we are here today.

With that as a backdrop, I would like to briefly speak to 5 specific points, and I of course will be delighted to answer any questions afterwards.

1. First I would like to speak to the opportunity before us to make a sustained and lasting impact. In many ways, we are witnessing the second great revolution in the
field of childhood cancers. While the 1970's and 80's were heady times when previously incurable leukemias, brain tumors and solid malignancies elsewhere the body were first cured, and cure rates increased to the incredible 80% rate we witness today, it is only in the last 5 years that we have begun to unlock the true underlying mysteries of why childhood cancers happen in the first place, and what are their Achilles' heels. Indeed, the rate of discovery is exponential, and opportunity to translate these discoveries into new ways to prevent, diagnose and treat childhood cancers is immense—I have never seen so much potential to make a real impact across the span of my career.

2. Second, I would like to emphasize the challenge of childhood cancers. Please note that throughout my testimony I have been careful to use the plural tense when speaking of childhood cancers. These very diverse diseases often get lumped together, but what we have discovered is that these are each unique entities, and there is not a "fix" for childhood cancer in general. However, we have made tremendous strides in certain diseases once we unlock the mysteries of particular types of childhood cancer. Perhaps the best example here at CHOP is the development of a new immunotherapy known as chimeric antigen receptor ("CAR") T cell therapy to treat relapsed acute lymphoblastic leukemia (ALL). While ALL is cured in the majority of patients with a three-year program of chemotherapy, it is typically incurable if it becomes resistant to chemotherapy. This new CAR therapy is curing previously incurable patients, and will soon become the first therapy of this type to receive FDA approval. However, while this has been a revolution for children with ALL, we have been stymied in our attempts to get this type of therapy to work in every other type of childhood cancer. We are still in the early days of immunotherapy for childhood cancers, but we have already learned many lessons in how brain cancers and other malignancies in children resist our attempts to harness the immune system. These lessons should redouble our efforts spanning the laboratory to the clinic.

3. Third, there are some very important facts, some of which are not well known to the majority of individuals in the Commonwealth. The memorandum that you have all reviewed very elegantly lays out some of the critical statistics regarding childhood cancers, emphasizing that it remains the number one disease killer of children. This is well known. While I mentioned previously the incredible increase in cure rates in several decades ago that impacted this reality, there are two very important facts that are under-recognized. First cure rates stopped improving around the turn of the century, and we have not made substantive improvements excepting in the CAR T program mentioned previously. Equally important, the cures we now have are based on intensive chemotherapy and radiation therapy. By the year 2020, there will be ½ million childhood cancer survivors in the US, and the vast majority of these individuals will be significantly burdened by the therapy they received as a young child. These so called "late effects" range from learning disabilities, heart failure, deafness, blindness, limitation in mobility and, most alarmingly, a very high rate of developing a new cancer as a consequence of our therapy. Thus, childhood
cancer is by far not a solved problem, and we need to etch in our minds that “cure is not enough”, but rather we need to strive for cure with quality.

4. This fact leads me to my fourth major point, namely the promise of precision medicine. CAR T cell therapy is a form of “personalized” or “precision” medicine, and this is the grand opportunity for pediatric oncology. Over the past few years, my own practice has morphed from every child with a particular diagnosis receiving the exact same therapy, to now providing a highly individualizing treatment to each patient, regardless of diagnosis, based on the precise genetic changes in that child’s cancer cells. We are now in an era where we can retrieve rare cancer cells and figure out every genetic mutation that is critical to make these cells want to grow, and then to target them specifically. We have a tremendous opportunity to make this the standard approach across the commonwealth, and ultimately across the globe, with the dual effects of curing more patients with much less collateral damage.

5. Finally, to make a true impact on the problem of childhood cancers requires a significant investment. I know two facts about the status of NIH funding for research in childhood cancer. First, there is currently not nearly enough money available. Second, it is very likely to go down. Those of us on the frontlines have met this challenge by writing more grant applications, diversifying our funding portfolios, working with childhood cancer foundations, and relying on our institutions clinical revenue. However, this is not nearly enough and each and every day of my professional existence is challenged by raising enough money to do the essential translational research. Indeed, my biggest fear is that we are going to lose our next generation of pediatric oncology researchers because it is simply too daunting to start and build a research program these days. This is exactly why this meeting, and the bills introduced by Tom Caltagirone, are so timely and so urgently needed for our four childhood cancer programs in Pennsylvania.

In conclusion, I would like to thank you Mr. Chairman, Representative Caltagirone and members of the Committee for this opportunity to testify on behalf of my colleagues at the Children’s Hospital of Philadelphia and the pediatric cancer research community. I look forward to answering any questions you may have.
Andrea Adams’ Testimony Submitted For The Record
House Democratic Policy Committee Hearing on Pediatric Cancer Research Funding
June 1, 2017

My name is Andrea Adams and my 14 year old son Juwan Adams is currently being treated for stage 3 Hodgkin’s Lymphoma. Juwan was diagnosed almost a year and a half ago. In that time he has undergone many cycles of chemotherapy, radiation and even a stem cell transplant. Right now, he is 97 days post transplant and is receiving additional maintenance chemotherapy. All of this is going on while he is trying to be a normal freshman in high school.

Prior to treatment, Juwan played baseball and basketball for his school. He also performed with the Montgomery County Honors Band, Abington Junior High School orchestra, Abington Ghosts Marching Band and Ghost Percussion Troupe as a drummer and percussionist. Juwan was a national honor society scholar and served on the executive counsel for a peer leadership group called Voices in Black Excellence. Juwan had a bright future ahead of him until the words “your child has cancer” altered our lives.

Additional funding for pediatric cancer is needed because so many children have died as a result of pediatric cancer. That is a given. It is devastating when you hear that a child has lost their battle with cancer. However, what about the children that’s currently fighting or better yet, living with cancer? When Juwan was diagnosed a lot of people said, “thank God he has a good cancer”. While no cancer is considered “good”, I understood what people, as misguided as they were, meant to say. Through the tireless efforts and research done so far, the medical community has been able to find treatments for cancers such as lymphoma and leukemia that weren’t available in the past. While hearing that your child has cancer is devastating, hearing that there are possible treatments gives us hope.

UNTIL...

We have hope until you go over your child’s roadmap (or treatment plan for those non-onco parents) and you realize that the side effects of the cancer fighting drugs include the possibility of a secondary cancer. You have hope until your star athlete can no longer run or walk because of the neuropathy and joint pain caused by treatments. You have hope until the night that your child has his first seizure and is afraid to sleep alone. You have hope until your previously independent and confident teenager is lying naked in your arms, sobbing because they cannot control their bowels or eat on their own. You then lose hope when your child with all of the potential in the world is declared cancer free but their body is so broken and beaten that they can’t walk up a flight of steps without getting winded.

For me this is why funding for pediatric cancer research is so vital. We need to save the children from dying but we also need to support the children that are living. The medical breakthroughs that are waiting to be discovered could mean that more children are able to fight cancer with more options and not with the same outdated treatments that have been used for decades.

Last night, Juwan was honored for all of those things I listed before as well as maintaining Distinguished Honors and his amazing community service efforts this year. His way of coping
with this terrible disease has been to reach out and help other children going through the same difficulties that he faces. While I sat in the audience and listened to all of the accolades that were bestowed upon him, I began to cry. While I am incredibly proud of my son I can’t help but think of what he could accomplish if he didn’t have to miss over 100 days of school for the past two years or deal with the PTSD and trauma of treatment and losing friends at such a young age.

What about the many children that will never have their potential realized because their bodies could not withstand the archaic treatments they were subjected to. Today I am dealing with a child that is depressed because he is not allowed to attend his Freshman Formal or class trip. He is angry that he is not allowed in school, his safe space, for another three months due to his compromised immune system. He is feeling guilty because he is still alive and many of his friends that he met during treatment are no longer with us.

I implore you to think about the future doctors, lawyers, writers, politicians, actors, etc. that we will lose if we don’t act now. Our children are depending on us. Their future depends on it. Act now! Awareness and funding for pediatric research is what can save them all. Thank you and God bless every family in this fight.