**Senate Finance Committee**
**Public Hearing – Senate Bill 74**
**September 25, 2019**

Good Morning. My name is Lisa McGregor and I am the chief of Pediatric Hematology-Oncology at Penn State Children's Hospital.

Thank you Senators Scott Hutchinson, John Blake, and members of the Senate Finance Committee for the opportunity to testify before you to discuss Senate Bill 74, and to provide my experience as a pediatric oncologist and some background on the current state of pediatric cancer research.

I'd also like to thank Senator Scott Martin for his introduction of Senate Bill 74 and the awareness this bill brings to the issue of pediatric cancer.

When I tell people that I am a pediatric oncologist, they often get a sad look on their faces and say "Oh, that must be really sad," or "I could never do that work." I can say honestly that for me it is the best job in the world. Taking care of these beautiful children, teenagers and young adults along with their families is a great honor and privilege.

Fifteen years ago, I started out as a newly trained pediatric oncologist. One of my first patients was a little boy, about 2 years old, with neuroblastoma, a cancer that started in one of his adrenal glands. It had spread to multiple bones in his body including his skull around his eyes. He was clearly in a horrible amount of pain from all the disease in his bones and he was losing his vision from the disease around his eyes. His mother was young and a single parent. She was very distressed, of course, at her son's suffering and seriously considered whether she wanted to make him sick with chemotherapy. In our discussions, I was very honest and told her that I expected the treatment available at that time to shrink his disease and relieve his pain but the reality was that only about 2-3 out of every 10 kids with cancer like her son's would survive more than 5 years without the disease coming back. She considered the options and ultimately chose to enroll her child on a research study looking at new treatments for neuroblastoma. As I predicted, he had a good response to the initial chemotherapy and lived for several years in remission before the disease returned about 7 ½ years after his initial diagnosis.

Since that time, the treatment of neuroblastoma has greatly improved. We have added several new therapies to the standard treatment and just 15 years later I am now able to tell a parent of a child with neuroblastoma that I expect 5-6 out of 10 to survive more than 5 years without the disease coming back. However, this improvement in outcome has a price attached. The treatment includes about six months of intense chemotherapy, surgery, two courses of high-dose chemotherapy with bone marrow rescue, radiation therapy, and six months of immunotherapy that is very toxic and painful. Most children who survive are left with one or more long-term health effects that may include problems with growth and pubertal development; infertility; hearing loss; dysfunction of the heart, liver and/or kidneys, and even second cancers caused by the treatment of the first cancer.

I am very proud of the improvement in outcomes that we have seen over the past 50 years and even over the 15 years that I have been practicing medicine. The pediatric oncology community has taken a group of diseases that caused the death of 4 out every 5 children diagnosed with cancer and switched the odds so that now 4 out of every 5 children survive. This great success has been the result of collaborations, across the US and the world, of scientists, physicians, and nurses. Most importantly, this success would have never been possible without the brave participation of patients and families like my first patient. Those parents who took a leap of faith and decided to enroll their child on a research trial in order to possibly benefit not only their child but children in the future have made the greatest difference in children's lives.

While we have seen great success and are able to provide our patients a more hopeful prognosis than in years past, we still lose 1 out of every 5 children diagnosed with cancer. Cancer remains the top cause of death from disease in children. We still have work to do until every child diagnosed with cancer has a long and healthy life. That work includes the following:

1. Research studying cancer cells and tumor models in the laboratory to better understand the cancers that happen in children. Childhood cancers are fundamentally different than cancers that occur in adults. Only by doing research dedicated to understanding how these cancers develop, grow and spread in children will we be able to specifically target these cancers with therapies that hurt the cancer cells while causing less collateral damage in the child's body. At Penn State Children's Hospital, some of our researchers work on the Ikaros pathway in high-risk acute leukemia. They have found that changes in the pathway are associated with poorer outcome. They are now developing a drug to target that pathway in the hopes of helping children whose leukemia has such changes. Hopefully one day this treatment will benefit many children.

2. Analysis of the genes and proteins in cancer cells taken from patients to identify what biochemical processes are active. Once we know what processes might be driving tumor development and growth, we can use targeted agents to pinpoint the specific pathways and halt cancer cell growth. Effective analysis of tumors requires state-of-the-art technology that is quite expensive as are the targeted agents, but with this approach we will be able to provide therapies that are personalized for each patient while decreasing toxicity to other tissues in the body.

3. Development of novel therapies such as those that target the blood supply to the tumor or recruit the patient's immune system to help cure the cancer. While the first two aims focus on the cancer cells themselves, it is important to understand that the cancer lives within a complex organism. In order to grow and develop it needs to develop a blood supply for oxygen and nutrients. When that blood supply is impeded, the tumors essentially starve. Developing the agents that impact the blood supply can be difficult as the tumors often find ways around the blockage but this approach has been effective against some cancers and further research may find ways to make the approach more effective. In addition, people have immune systems that often identify abnormal cells and eliminate them. Cancer cells must find a way to evade the immune system in order to continue to exist and grow. There are drugs in development that either uncloak the cancer cells for immune destruction or train the immune cells to identify the cancer cells. The latter, known as CAR-T cells have been effective for acute lymphoblastic leukemia and some lymphomas. With further development, this approach may be effective against many other cancer types.

Developing new treatments takes time and financial support. The path from laboratory experiments all the way through to clinical trials requires large amounts of resources. Because childhood cancer is rare, developing therapies specifically for children is not always profitable and thus is not at the forefront of the business strategy for pharmaceutical companies.

I hope that I have effectively described for you today the success that we have seen in treating children with cancer. We have shown that improving treatment is possible, but the current state is not good enough. If we as a society want to see continued improvement in the survival and the health status of the survivors of childhood cancer, we need to invest in the research to push the field forward. Pennsylvania has been at the forefront of the success and has the opportunity to continue to lead the nation with bills such as Senate Bill 74.

Again, thank you so much for this opportunity to speak with you and explain why this bill would help the children and adolescents of our state.