STATEMENT OF
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AND
GOVERNMENTAL AFFAIRS
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Connecting Patients to New and Potential Life Saving Treatments

Thank you Chairman Johnson, Ranking Member Carper, and Members of the Committee for inviting me here today. I am honored to testify before you about how to connect patients to new and potential life saving treatments.

I am the Executive Director of Kids v Cancer. But more importantly, I am the mother of Jacob, a very sweet, beautiful boy who died when he was 10 of a pediatric brain cancer. Despite the remarkable developments in cancer research, the drugs and protocols used to treat Jacob were 40 years old. The day after Jacob died, I launched Kids v Cancer to focus on changing the landscape of pediatric cancer research and to make it possible for children to get access to cutting edge new treatments.

When Jacob was in end stage cancer, I contacted eight different companies, requesting access to their unapproved drugs for Jacob. Finding the right person to contact was very difficult. The companies did not have point of contacts on their webpages, SEC filings or other public outlets. For some companies, I contacted the CEO, others the CMO, other the head of business development. For one company, it was my cousin's friend. It was all very *ad hoc*. The process was confusing and took me away from my son. Of the eight companies, six never got back to me. Two formally considered my request and declined.

The purpose of the Right To Try laws is to help patients get access to drugs they would not otherwise get access to. That is a serious problem, and that is a goal I share, but I think we need to take a broader approach to this problem and that has been the focus of Kids v Cancer.

Kids v Cancer's first step was to incentivize companies to develop drugs specifically for pediatric cancers and other pediatric rare diseases. In 2012, Congress passed the Creating Hope Act as part of PDUFA. That has created nearly $800 million in market incentives – at no cost to the taxpayer – for companies that get a new drug for pediatric cancer approved by the FDA. The Creating Hope Act is up for renewal as part of the 21st Century Cures bill passed by the House. I urge the Senate to renew it as well.

Our second step was to make news drugs being developed for adult cancers available for kids as well. In 2003, Congress passed Pediatric Research Equity Act (PREA), which requires companies developing drugs for adults to conduct pediatric trials on such drugs where it could benefit children. The problem is that PREA has not kept up with the science. PREA only requires clinical trials if the children have the same "indication" – that is, if children have the same type of cancer. But now we know that even though children don't get breast cancer or

lung cancer, the mechanism of action in these cancers might also be evident in pediatric cancers such as neuroblastoma or medulloblastoma, the type of brain cancer Jacob suffered from.

We have a proposed the Kids Innovative Drugs Initiative, a modest change to PREA that would update it to take into account these new scientific developments and ensure that drugs being developed for adults that could have relevance to pediatric cancers are tested on children as well. I urge Congress to pass the KIDS initiative as soon as possible, either as part of the 21st Century Cures bill or as part of PDUFA.

And that brings me back to Right to Try laws. Yes, when it comes to seeking compassionate use access to unapproved drugs, the paperwork is onerous and the process time-consuming. In response, Kids v Cancer is launching a Compassionate Use Navigator. We are working to better inform physicians on how to apply for compassionate use applications for their pediatric cancer patients with drug companies, the FDA and their hospitals. We hope to provide point of contacts for drug companies, we will post the new FDA expanded access form, and we will work with the institutional review boards of the hospitals where the patients will be treated. We will offer to counsel physicians personally on specific applications. In addition, we will collect information about the efforts and outcomes of pediatric cancer compassionate use applications.

The Compassionate Use Navigator is not the whole solution to the challenge of access to new treatments. However, it will give parents of dying children more time with their kids. It will lessen the burden of their physicians as they apply for compassionate use applications. We hope it will encourage more physicians of kids with cancer to apply for compassionate use. And, we hope this program will eventually lead to more children gaining access to compassionate use drugs.

In addition, Kids v Cancer supports the Andrea Sloan CURE Act to have drug companies make available to the public their policies on requests for compassionate use access, including the minimum criteria for approving requests and the time needed to make a decision. I urge Congress to pass the Andrea Sloan CURE Act as part of the 21st Century Cures bill.

But from my personal experience and from working with dozens of other families, my sense is that the fundamental problem is not the FDA, but the incentives faced by the companies. Even though the FDA approves virtually all compassionate use applications it receives, and even though it has indicated that an adverse reaction to a drug provided for compassionate use will not adversely affect a company's application for that drug's approval, companies remain risk averse and would rather not provide such drugs.

But even if one could change that, the results would be one-off anecdotes. We cannot afford to take an *ad hoc* approach to addressing pediatric cancer, the number one disease killer of children in America. We need to address the lack of access seriously ill children have to novel, unapproved drugs not only by one-off compassionate use applications, but even in clinical trials. That's why initiatives such as the Creating Hope Act and the KIDS Initiative are so important.

Any bill that makes it easier for children to get access to drugs they need to survive and live happier, healthier lives is, of course, welcomed, but we need to do more than address anecdotes. We need to change the landscape of pediatric cancer research. We need to ensure that children with cancer and other life threatening illnesses, like my son, Jacob, have access to new and potentially life saving treatments.

Thank you very much.