THECURESTARTSNOW®

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Thank you Chairman Casey, Ranking Member Braun and distinguished members of the Committee for the opportunity to discuss the Promising Pathway Act, something I consider vital to our efforts to find the "homerun cure" for cancer.

As you stated, I am a father. My daughter, Elena, fought a cancer called DIPG brain cancer when she was 6-years-old. Like thousands of families before us, they told us we had no hope, there were no trials available and to go home and make memories. Sadly, she understood this as well, fully aware until her last day that there was nothing we could do. You see, with DIPG, each day you see your children lose something. One day it was he ability to see out of her left eye, the next her ability to speak, then her ability to walk, until one day it is her ability to breathe, swallow or her heartbeat. Yet the brain is untouched in a particularly cruel cancer that progresses within weeks. Many experts regard this as the "cancer they fear most." Other experts see in it the chance to advance strategies like no other cancer does. Universally it is known as a "homerun cure" cancer.

We wrote about this one day during her battle in a blog ending simply with the words "the cure starts now" and with it started a cause as we were joined by hundreds of families and researchers that changed the course of their careers. Contrary to our wishes, this blog became a bestselling book translated to 22 languages, leading to the calling that my wife and I inherited. We have made it our mission to focus on these homerun, rare cancers and by building tools such as linked registries, intelligent patient tools, strategic research, centers of excellence and virtual hospitals that today are being used as models for other cancers. In the nearly 17 years since she lost her fight, we have partnered with 129 hospitals in 17 countries to invest in over $30 million in research and support through 135 projects.

I come here with hope. Hope for a new way to fight cancer. I believe that the FDA is well-intentioned, but in the end, no one expects this type of devastating cancer – let alone afflicting your children. Instead, I believe there is much confusion inherited from bureaucracy. And whether by regulation or guidance clarity, I argue that the system is broken and the status quo will not do.

It is important to note that Expanded Access, or Compassionate Use is not an alternative for the Promising Pathway Act. I know this not only from dealing with the loss of my daughter but also in calls with over 200 families per year asking for help in their final days.

With DIPG, a patient is frequently given only two weeks after progression to act. Expanded Access requires four levels of approval:

- 24 hours for the family or patient to react to the news
- 3 days for the doctor to determine a strategy and complete an application

- 2 weeks for the Internal Review Board to approve the methodology
- 3 days for the pharmaceutical company to respond
- 1-30 days for the FDA to render approval

That's at least a week after our child dies.

Expanded Access and Compassionate Use is a "wild west" strategy to medicine sadly predicated on your doctor's ability to craft a bureaucratic narrative to satisfy both their IRB and the FDA.

Then there is Right to Try. Slightly better with a two-level approval process, it still lacks data transparency to patients and families in an annual report model that only submits to the FDA.

The current models just don't work. They assume that "terminal" means "months to live", they assume patients are not intelligent enough to make decisions for themselves, they assume that data in less than ideal situations are worthless, and they assume that every patient is an adult.

The reality is that we don't get doctors that understand the system, we don't get a call back on the weekend and not one of these acts were designed for pediatric terminal diseases.

The Promising Pathway Act is different.

- It offers an alternative to the FDA-mandated unethical practice of trials giving the wrong drug to the wrong patients for pediatric terminal diseases. It is estimated that 1,040 children die each year from cancer. Right now, nearly every therapy designed for pediatric cancer is guided through a three-phase adult trial before we get to try it on terminally diagnosed children. Sadly enough, some of these cancers don't even affect adults, meaning that we either misapply a trial on the wrong patients or, worse yet, the drug company simply gives up. Now remember that some of our biggest advances in cancer started with pediatric cancer. You want to know why we can't advance a cure? This is why.
- It preserves critical data on outlier participants in a trial and retains it for the use by the FDA, the drug companies, the researcher AND the patients - in any third-party registry of substance, thus improving transparency.
- It addresses insurance coverage and financial burden in ways neither previous alternative has.
- It advances innovative therapies that may be impossible to subject to current trial design. Keep in mind that trials for "rare" cancers "rarely" ever achieve full accruals, leading us to either give up on good ideas or enrolling children in older trials that we already know don't work. PPA could be another model on how we could save countless lives.

PPA is not for all patients. The language is careful to consider the definition of "terminal" and does not create a shortcut to trials as it may be alleged.

It is also not lost on me the value of a clinical trial. But what is often lost on many others is the value of a QUICK and EFFICIENT clinical trial. Unfortunately, with rare pediatric cancers, we have to consider both this, and sadly in their final days – we also have to consider compassion. At any point there are roughly 200,000 people fighting a terminal disease. For them, PPA could be a lifeline to treatments they may never see before they die.

I'm a big believer in personal initiative but in this case, we may be able to go NO FURTHER unless government gets out of the way. The Promising Pathway Act is how we do this. Families are desperate for another chance: a chance at seeing their children go to kindergarten, a chance to see their child get a driver's license, a chance to see them graduate from high school, a chance to see them go to college, a chance to see them get married – a chance to see them grow up.

PPA may be the single biggest piece of legislation that costs nearly nothing, but may change everything about how we win against cancer.