Creating Hope:
In Support of the Creating Hope Reauthorization Act, Making the Rare Pediatric Disease Priority Review Voucher Program Permanent

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My name is Nancy Goodman. I am here as Founder and Executive Director of Kids v Cancer, a non-profit organization that has been the driving force of the Creating Hope Act, which established the rare pediatric disease priority review voucher program, as well as the driving force behind the RACE for Children Act, which requires novel cancer therapies to be studied in children's cancers. Working with the pediatric cancer and rare disease community and, very importantly, with Members on this Committee and others, we have made significant progress in changing the landscape of pediatric drug development.

Among other initiatives, Kids v Cancer promotes legislation that is in the best interest of seriously ill children, regardless of whether the pharmaceutical industry supports or opposes it. Towards that end, we do not accept donations from drug companies or their employees. I have drafted and am solely responsible for the following comments.

I am here not only in my capacity as Executive Director of Kids v Cancer, but also as mother. More than a decade ago, I lived in Manhattan with my husband and my sons, Jacob and Benjamin. When Jacob was eight years old, he began waking up with nausea and headaches. We went to our pediatrician's office one Sunday morning. Sitting on an examination bed, wearing a Micky Mouse tie, our pediatrician told us Jacob might have a brain tumor.

We soon learned Jacob had medulloblastoma, a rare pediatric cancer affecting fewer than 500 American children a year. The drugs available to treat Jacob were 40 years old. There had been very little innovation in the standard of care. And what was available was unlikely to work.

How could that be, I wondered, when there were so many exciting leaps forward in treating adult cancer therapies? Was there really no path forward to develop new treatments for the tens of thousands of children who are diagnosed each year with unique children's cancers and other rare pediatric diseases?

Jacob died in the early hours of a wintry Friday morning. He was 10 years old. On Saturday, I founded Kids v Cancer.

We started with drafting the Creating Hope Act, which creates a pediatric priority review voucher, based on the tropical disease voucher, to provide a market incentive for companies to develop *de novo* pediatric rare disease drugs, including pediatric cancer drugs. It was

The challenge we sought to address was that there were inadequate market incentives to develop drugs for small numbers of children suffering from rare diseases.

As most rare pediatric disease drug development is conducted by biotechs, we wanted an incentive largely funded by major pharmaceutical companies that would make transfers to smaller biotechs in the form of payments for the pediatric vouchers biotechs earned.

We did not have drafting experience, FDA expertise or lobbying experience. We had Jacob's friends, his little brother, Ben, and hundreds of other children touched by cancer who came to Capitol Hill to tell you their stories. Working closely with Members of this Committee and others, the Creating Hope Act was passed into law in 2012 as 21 USC 360ff. Thank you.

After Congress passed the Creating Hope Act in 2012, we turned our attention to the thousand-drug pipeline of therapies in development to treat adult cancers. Only a handful were also in development for children's cancers. We drafted a new bill, the RACE for Children Act, which would modify the Pediatric Research Equity Act. The RACE for Children Act requires pediatric studies to be undertaken for each oncology drug in development when the molecular targets of the drug are substantially relevant to one more pediatric cancer indications. Again, we employed hundreds of children and young adults to come to Washington. And again, working closely with Members of this Committee, in 2017, Congress passed the RACE for Children Act.

I am proud to state that RACE for Children Act will be fully implemented on August 18, 2020. However, on September 30, 2020, the pediatric voucher program is scheduled to expire. Since the passage of the Creating Hope Act in 2012, the pediatric voucher program has approached a sunset date and been reauthorized three times. Now, to address this fourth sunset date, the Creating Hope Reauthorization Act (HR 4339) permanently reauthorizes the pediatric voucher program. I am here today, respectfully asking you to pass this bill.

The pediatric voucher program has been successful and has created a pathway for small biotechs and even larger pharmaceutical companies to develop drugs for pediatric rare diseases.

The first measure of success of the pediatric voucher program is that the program has mobilized well over $1 billion in incentives for pediatric drug development in the form of payments for vouchers. These vouchers are market incentives that promote pediatric drug development at no cost to the American taxpayer or consumer. A company that gets approval for a specific pediatric cancer or rare disease drug receives a voucher, which is transferable and which entitles the holder to put another drug, perhaps a major blood pressure drug, through the FDA's priority review process, potentially allowing them to get to market more quickly. Pediatric rare disease vouchers have traded for between $67 million and $350 million. They

have recently been hovering just under $100 million, creating opportunities for market-based, risk adjusted returns on investment in rare pediatric disease drug development.

A second measure of success is the increase in the number of new drugs that have been approved for rare pediatric diseases before the passage of the Creating Hope Act and after. A recent study by identified 15 drugs that could have potentially earned pediatric vouchers in the 12 years prior to commencement of the pediatric voucher program and identified 22 rare pediatric disease drugs that were approved after passage of the Creating Hope Act, a jump in FDA approval of rare pediatric disease drugs of over 120%.

A third measure of success is the increased number of rare pediatric disease drugs that are in the drug development pipeline. One way to track this are by the number the rare pediatric designations issued by the FDA, which provide that a drug in development, if approved, merits a pediatric voucher, may be issued early in the development process. In 2013, the FDA granted rare pediatric designations to only a handful of drugs. In 2016, the agency granted 24 designations. In 2019 the FDA granted 64, a more than 200% increase in the number of rare pediatric diseases in development in since 2016.

But perhaps the most important, long-term impact of the Creating Hope Act has been to change the risk/return calculation of biotech and pharmaceutical companies such that they are increasingly interested in pediatric rare disease drug development. At the time of Jacob's illness, there were no pediatric cancer drug development companies. Now, with the pediatric voucher program's influence, there are several. And many more companies are now willing to invest to pediatric rare disease drug development along with other products than there were before.

The Creating Hope Act has been a success, but its short duration has created uncertainty has been a challenge. Since 2012, the program has been reauthorized three times, each time for only one to four years. However, the time horizon for drug development from idea to FDA approval is 7 to 10 years and often longer. Because researchers and executives at the start of a drug development plan cannot count on the existence of the pediatric voucher program by the time their drug might be approved, they are less likely to be incentivized to develop rare pediatric disease drugs.

If pediatric voucher program is reauthorized on a permanent basis, as directed in the Creating Hope Reauthorization Act, one can expect the voucher program will have an even greater impact on the number of new rare pediatric disease drugs in development.

As you know, drug development is expensive. A new drug may cost tens or hundreds of millions of dollars to commercialize, as it goes from bench science to FDA approval. Federal funding mechanisms for rare pediatric disease drug development of this magnitude do not exist. Small Business Innovation Research, SBIR loans available to drug developers are in the range of $1 million. The National Cancer Institute's entire budget for pediatric cancer research is around $250 million, making it impossible to issue a grant of $50 million to commercialize

one pediatric cancer drug. Few foundations have endowments large enough to fund their own commercialization of novel therapies. There certainly is not such a foundation dedicated to medulloblastoma or any number of other rare pediatric diseases.

There could well be other approaches to promote pediatric rare disease drug development, but each has its downsides.

Whereas tax credits and other tax incentives create a burden to the taxpayer, the pediatric voucher program creates no cost to taxpayers. Instead, vouchers constitute a transfer of value generally from larger pharmaceutical companies that wish to exercise vouchers to bring drugs to market more quickly, to smaller biotechs that are developing rare pediatric disease drugs.

Let me repeat that: The Creating Hope Act basically facilitates a transfer of value from large pharmaceutical companies to smaller and, often times, more innovative biotech companies to facilitate their investment in creating drugs for pediatric cancer and rare diseases which they would otherwise not have a commercial incentive to pursue. It is solving for a market failure.

Moreover, the pediatric voucher program creates no additional costs to consumers. Unlike the Best Pharmaceuticals for Children's Act award of six months pediatric exclusivity for negative trials, and unlike the extended exclusivity of the Orphan Drug Act, the pediatric rare disease vouchers do not extend exclusivity and, therefore, do not delay lower prices for consumers provided by generic and biosimilar competition.

The Creating Hope Act has increased the number of reviews in the FDA's priority review program. That is why, when originally drafting the Creating Hope Act, we were careful to include a provision by which the FDA could compensate itself for any additional burden by allowing the FDA to set voucher user fees. These fees would reflect the cost to the FDA of the voucher program and could be reset annually if the FDA determine they are inadequate. In 2020, drug manufacturers exercising a voucher pay a $2.16 million fee to the FDA for priority review on top of a $2.4 million user fee they would owe for any drug application. If there are remaining issues around the management program, I encourage FDA leadership to address them head-on rather than abandon a program that promises hope to so many critically ill children and their families.

We are at a historic moment in global public health as COVID infects so many. Since the beginning of COVID, around 100 children have died of COVID, which is a tragedy. However, since the beginning of COVID, thousands of kids have died of cancer and other rare diseases. So, even as we take all look to take steps to create new COVID therapies and vaccines, can't we also come together to save the lives of children with cancer and other life-threatening illnesses? All available evidence indicates that the pediatric voucher program is an effective program in doing so. I urge you to support its permanent reauthorization by passing the Creating Hope Reauthorization Act.

Thank you for including me in today's hearing. I am happy to take your questions.