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Prepared Comments to
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Thank you all for inviting me to speak to you today about the Creating Hope Act, a law that provides market incentives for the development of drugs for children with rare diseases.

I want to begin my presentation as I always do, by talking about my son, Jacob. Before he was diagnosed, Jacob was a beautiful eight year-old boy with red hair and a brilliant, kind smile. He loved watching older boys play basketball in Riverside Park. He loved to sing Black Eyed Peas songs. He loved to put on Broadway musicals in the living room with his little brother.

After he was diagnosed -- and subjected to treatment -- Jacob suddenly became a boy in a wheelchair. A boy … who at times couldn’t talk or even move his limbs … who could neither eat nor control his basic bodily functions. A child who spent 9 of the next 23 months of his life in hospitals and much of the rest in oncology clinics.

Two weeks after Jacob’s diagnosis and only days after a partial resection of his widely metastasized brain tumors, Jacob’s doctors started the standard protocol of radiation and chemotherapy. Jacob’s medical team knew pretty early on that the drugs and radiation were unlikely to work. Yet they continued on course because there were no alternatives. In fact, there had been no significant changes in the protocol for decades. Why is that?

Jacob died on a Friday night. Saturday, I opened up my laptop on the dining room table and founded Kids v Cancer. I’m here today to thank you for also choosing this journey.

The adult cancer pipeline has almost 900 drugs in it. The pediatric cancer pipeline? It’s so small that few of us know much about it.

Of the 900 drugs, a handful will eventually be approved for an adult cancer and have efficacy for a children’s cancer. However, many of those 900 drugs will fall out of the pipeline when they do not demonstrate efficacy in the adult cancer for which they are being developed. They

will be abandoned before they are ever tested, even preclinically, on children's cancers. This is a crisis.

Lobbying for the Creating Hope Act

So, our first order of business was to create a market incentive for companies or universities to develop drugs for expressly for kids. – and also for companies not to abandon adult cancer drug development if there is hope that a drug could have efficacy in kids.

We started with the United States Congress, drafting a bill we called Creating Hope Act. I had never lobbied Capitol Hill before. I went from Congressional office to Congressional office, perhaps 500 meetings. We garnered 172 cosponsors both Republicans and Democrats, hosted lobby days, launched a social media campaign and held lobby days, generated thousands of emails and phone calls, and attracted a lot of positive press coverage.

I'm very happy to tell you that the Creating Hope Act passed and was signed into law last summer, as part of the 2012 FDA Safety and Innovation Act, codified as 21 USC Section 360ff.

The best lobbyists we had? Jacob's friends and his little brother, Ben. They could make the case for action like no one else. I can't tell you how many meetings ended with tears and hugs from cynical Congressional staffers and case-hardened Senators.

But the Creating Hope Act is not about sympathy or pity. It's about hard-headed economics – making doing the right thing profitable by any measure.

Summary of the Creating Hope Act

Under the Creating Hope Act, a company that achieves FDA approval for a pediatric rare disease drug it has developed is awarded a voucher, fully transferable.

The voucher entitles the company to priority review (which is similar to the EMA's accelerated assessment) of another drug.

Thus the company could get that second drug - - perhaps a large market drug -- to market faster. It would also give the drug an opportunity to beat a similar competing drug to market, thereby generating significant value.

I'm sure most of you today understand what an advantage that creates – and thus, what a potentially powerful market incentive a Creating Hope voucher could be to develop pediatric cancer drugs.

This is an exciting concept. No one knows for sure at this point what the value of a voucher is. Analysts and economists have generated estimates from tens of millions of dollars on up. But whatever its magnitude, the value of a voucher would fall almost entirely to a company's bottom line, as the cost of pursuing a voucher is minimal.

It could help fund research into new lines of drugs or help a company mitigate risk while diversifying its offerings. And it could help demonstrate that investment in pediatric rare disease drugs can be justified purely on financial returns. Right now, we are now working with companies and research institutions with drugs that could qualify for vouchers.

By June 2012, four applications for Creating Hope Act designations had been filed with the FDA.
I'm told there are more now. The FDA has authorized at least one voucher. And, many more companies are posed to apply for designations as soon as the application for designations is issued.

Finally, we look forward to the publication by the FDA of a draft guidance and draft designation application form in Spring, 2014.

Legislative Details of the Creating Hope Act

I want to take a moment to go over in more detail the mechanisms of the Creating Hope Act.

Let's take the case of a company developing a pediatric cancer drug.

a) Designation process

The first step for the company would be to apply for a Creating Hope Act designation that the

pediatric cancer drug if approved, would qualify for a voucher.

The application for a Creating Hope Act designation could be made as early as the time a request for a designation of orphan drug status is made. The application process is not burdensome and incorporates the steps of the orphan designation.

It has two requirements:

- first, that the indication for which the drug is being developed qualifies as a "rare pediatric disease" and
- second, that the drug itself qualifies as a "rare pediatric disease product application."

To be a rare pediatric disease a disease

- primarily affects individuals aged from birth to 18 years;
- it must have an orphan designation.

For a drug product to be a Rare Pediatric Disease Product Application, the drug must be for

the prevention or treatment of a Rare Pediatric Disease
It must be a new molecular entity or new chemical entity
It must rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for a pediatric population; and
It must go to the FDA for approval first on a pediatric indication

We incorporated a designation process into the Creating Hope Act to enable companies developing pediatric rare disease drugs to have an early signal that they were on the right path to a voucher.

We believe this will create benefits to the company in terms of investor relations, greater market value, smoother financing, and an easier exist strategy.

b) Approval of the pediatric cancer drug and a voucher

Once a designation has been achieved, the next step is for the drug to be submitted to the FDA for approval. If approved, then the FDA also grants the drug company a voucher.

c) Exercise of the voucher

The next step is the exercise of the voucher.

The drug company that wishes to exercise a voucher would likely have a large market adult drug such as an adult cancer drug or perhaps a new statin for which it is submitting an application for approval.

The Creating Hope Act does not require that the company exercising the voucher be the same company as the one that earned the voucher. In fact, the vouchers have unlimited transferability.

The drug company planning to exercise a voucher must notify the FDA of its intent to submit a drug application with a voucher at least 90 days prior to submission.

In addition, at the time of the notification, the drug company musy pay a user fee to the FDA. The user fee is intended to reimburse the FDA for the extra expense of undertaking a priority review.

d) approval

The voucher program does not alter the criteria for a FDA approval. Drugs subject benefiting from a voucher must still conform to all the FDA requirements to be approved.

e) marketing requirements

Finally, the Creating Hope Act has a post approval marketing requirement that the rare pediatric drug product drug is marketed in the US within 365 days.

The Creating Hope Act is a pilot program of three vouchers and as many vouchers as are created in the 12 months following the creating of the third voucher. Like other pediatric legislation in the United States, the Creating Hope Act will have to be reauthorized by Congress to achieve permanent status.

What are the Implications of the Creating Hope Act for the EMA?

Because pediatric cancer markets are small, pediatric drug development may not always be global. A company with a promising investigational pediatric cancer drug may not submit it to both the FDA and the EMA for approval.

The incentive of a Creating Hope Act voucher in Europe could prompt companies to pursue approval and authorization to both regulatory agencies – assuring US and European children alike access to effective new therapies.

Nuts and bolts:

To think about what the value of a voucher might be in Europe, I would like to refer to a 2010 Lancet Article by Duke Health care Economist David Ridley. Ridley writes on a voucher program for tropical diseases, the program that the Creating Hope Act was based upon.

We have found that the voucher is a more powerful tool for pediatric rare diseases than for tropical diseases because it does not change the cost structure of drug development for pediatric diseases. However Ridley's analysis of tropical disease drug development still provides an example of how the Creating Hope Act could be grafted to Europe.

Instead of relying upon a priority review program, Ridely proposes that the value of an EMA voucher could be derived from:

- accelerated assessment marketing authorization and also from
- accelerated pricing and reimbursement procedures.

Ridley proposes that a drug benefiting from the exercise of an EMA voucher would first be granted access to the centralized approval process, even if it was slated for the national approval process. Then, the drug would receive an accelerated assessment.

Ridley also proposes accelerated pricing and reimbursement decisions in EU Member States because unlike in the US, pricing and reimbursement questions in Europe often do not resolve quickly and because they involve governmental entities. Ridely further proposes that a manufacturer with a voucher could still choose to pursue pricing and reimbursement sequentially in different countries.

I am not an EU regulatory expert and did not undertake a complete analysis of a European application. I included this more as a demonstration of what a European regulatory framework might look like.

Wrap up

Let me end where we began. We are at a critical inflection point in pediatric drug development.

We have an explosion of research about targeted therapies.

We have new tools for translating the progress being made on adult cancers to pediatric cancers, and to increasing access to promising drugs for pediatric cancer researchers.

We could take the incentives we have created to keep drug development of abandoned drugs alive. To you in industry, please give us, give me, an opportunity to acquire the IP and clinical supply. We will assume development and marketing responsibility. Let's save some kids.

Thank you for inviting me here today to share my thoughts with you

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