

**PRESENTATION TO BIO CEO FORUM
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Thank you for joining me to discuss an important market incentive for the development of rare pediatric disease drugs, specifically the Creating Hope Act, enacted into U.S. law as 21 USC 360ii, the Rare Pediatric Disease Priority Review Program.

I began working on this issue five years ago when my 10-year old son, Jacob, died of a pediatric brain cancer, medulloblastoma. Jacob lived for two years after diagnosis, undergoing treatment after treatment, much of it in discomfort, none of it effective.

We think of drug development in cancer as an exciting, robust area, but there had been no significant changes in the protocol for Jacob's form of cancer in decades.

The morning after Jacob died, I opened up my laptop on the dining room table and founded Kids v Cancer. Our goal has been to change the landscape of pediatric cancer drug development. And I'm here today to invite you to join me in this effort and to share a potentially powerful incentive for you to do so.

In this era of fiscal constraints, I knew it was a waste of time to try to mobilize significantly more Federal funds for pediatric cancer research, so we worked to create a market incentive for biotech and pharmaceutical companies to develop drugs especially for kids with cancer and other pediatric and rare diseases. We started with the United States Congress, drafting a bill we called Creating Hope Act and that was passed into law as part of the FDA Safety and Innovation Act.

Under the Creating Hope Act, a company that achieves FDA approval for a pediatric rare disease drug or biologic it has developed is awarded a voucher, fully transferable, which entitles the holder of the voucher to priority review of another drug. This would enable a company with a large-market adult drug, for example, to get to market more quickly than it would under the standard review procedure. It would also accelerate the revenue stream and create a longer marketing period covered by intellectual property.

I'd like to walk you through the details of this program for a few minutes.

The first step in Creating Hope Act program is a designation that the drug, if approved, it would qualify for a voucher.

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 a promising path to a voucher. We believe this will create potential valuation and financing benefits for the company.

The application for a designation could be made as early as the time a request for a designation of orphan drug status is made.

The designation process has six requirements:

1. The diagnosis primarily affects individuals aged from birth to 18 years;
2. The diagnosis has an orphan designation.
3. The drug or biologic must be a new molecular entity or new chemical entity
4. The NDA/BLA must use clinical data and dosages/scheduling based on a pediatric population;
5. The drug/biologic must go to the FDA for approval first on a pediatric indication
6. The drug/biologic must receive priority review itself

The next step in the Creating Hope Act program is for the drug/biologic to be submitted to the FDA for approval. If the drug or biologic is approved, then the FDA grants the sponsor a voucher.

The sponsor that has received a voucher may subsequently use it on another drug, perhaps a large market drug that would otherwise receive a standard review.

Or if the sponsor does not have a drug/biologic for which it would choose to exercise the voucher, it may sell the voucher. Vouchers may be transferred an unlimited number of times.

The company intending to exercise a voucher must notify the FDA of its intent to submit a NDA/BLA and exercise a voucher 90 days prior to submission.

In addition, at the time of the notification, the company must 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 instead of a standard review for a drug that does not, on its own, merit a priority review. The user fee for 2014 is about \$2.3M.

The Creating Hope Act program includes a post approval marketing requirement that the rare pediatric disease drug product is marketed for the US within 365 days.

Congress passed the Creating Hope Act as a pilot program. The FDA is authorized to grant three vouchers and as many vouchers as are created in the 12 months following the third voucher. Like other pediatric legislation in the United States such as BPCA and PREA, the Creating Hope Act will have to be reauthorized by Congress in order to be made permanent.

The Creating Hope Act is based on the tropical disease priority review voucher program and has several critical changes. First, the Creating Hope Act voucher is fully transferable. The tropical disease voucher is transferable only once. Second, the period of notice to the FDA before exercising a voucher was decreased from a year to three months. Third, the Creating

Hope Act includes a designation process to give companies greater certainty as to whether they are likely to receive a voucher.

Thank you for inviting me here today and for joining me. For pharmaceutical and biotech companies, and for the right set of investors and philanthropists, the Creating Hope Act presents an opportunity to demonstrate that good, risk-adjusted returns can be made by investing in pediatric drug development. In other words, that you can do well -- and do good -- at the same time.

Kids v Cancer is committed to working with you towards this goal. We look forward to working with all of you to make this a reality.
