

Kids with cancer need access to drug trials

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Friday, 21 Nov 2014 | 6:00 AM ETCNBC.com

Parents who have lost children to cancer and young survivors of cancer are tied in a terrible and close-knit tribe. I am a member. My son, Jacob, died of a pediatric brain tumor when he was 10. Some parents and survivors focus on awareness by cloaking iconic monuments in gold or they make beautiful art. I chose to honor Jacob by changing the rules of the game.



Amy Bolger
The Froman family, including Nancy Goodman, Mike Froman, and kids Ben and Sarah. They hold a photo of Jacob, who died of medulloblastoma, a rare form of brain cancer, at age 10.

The greatest hurdle in battling childhood cancer has long since been the lack of treatments developed specifically for children. Out of 900 drugs in the cancer pipeline, almost none are for kids. It's a matter of market failure.

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When Jacob died, I started thinking about how we could encourage companies to develop drugs specifically for children with cancer. I learned of a new program of priority review vouchers that was initially created to provide faster reviews by the FDA as an incentive for pharmaceutical companies to invest in new drugs and vaccines for tropical diseases. I founded Kids v Cancer and together with the pediatric cancer community, championed the Creating Hope Act, which extended the opportunities of priority review vouchers to include rare pediatric diseases, including pediatric cancer. In 2012, we got it passed into law.

Under the Creating Hope Act, a company that develops a drug specifically to treat a rare pediatric disease, including pediatric cancer, would receive a pediatric priority review voucher, which the company could use or sell. The voucher entitles the holder to a faster review of another drug by the FDA. The theory was that the voucher could be valuable to companies wanting to get a drug to market quicker and, therefore, create incentives for companies to engage in pediatric drug development.

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The good news: The theory worked.

In July, we saw the first transaction when [BioMarin](#) sold a Creating Hope Act priority review voucher for \$67.5 million. This deal provided proof that good, risk-adjusted returns can be made by investing in pediatric drug development.

This week, [Knights Therapeutics](#) sold a tropical disease priority review voucher to [Gilead](#) for a whopping \$125 million, almost twice the value of the first voucher.

These deals validated our strategy.

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We have reached a first tipping point in pediatric drug development. Where it was once financially unfeasible, businesses now have a good, bottom-line oriented reason to develop drugs for kids with cancer and other rare diseases.

This is a good first step, but there is more to do so that fewer children follow in Jacob's dark footsteps.

Commentary by Nancy Goodman. She is founder and executive director of Kids v Cancer and champion of the Creating Hope Act, which established pediatric priority review vouchers as an incentive for pediatric rare disease drug development. Follow her on Twitter@KIDSvCANCER and on Facebook at Facebook.com/kidsvcancer.

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