# How to correct the market for children's cancer drugs

The development of pediatric cancer drugs is vastly underfunded compared with that for adults, but legislation can correct market failures.

Everyone cares about children with cancer. Photos of smiling, bald kids elicit an instinctual, emotional reaction. Those kids deserve to live full and healthy lives. All of us are willing to sponsor telethons and run road races to make that happen. But legal and regulatory systems designed to create incentives for pharmaceutical and biotech companies to develop drugs are not also designed to incentivize drug development for children with cancer. That has begun to change, but we have a long way to go.

My son, Jacob, could have been in one of those photos. At eight, Jacob was a boy whose passions were singing 1990s rock songs and playing baseball. One morning, he began to experience nausea and headaches. Soon after, our world fell apart as Jacob was diagnosed with medulloblastoma, a pediatric cancer. I was stunned by the diagnosis, and I was shocked to learn that the cancer therapies available to treat Jacob were over 40 years old, and were unlikely to work. Jacob died two years later.

The gap between the value society places on the health of children and the lack of resources it dedicates to pediatric cancer drug development arises due to market failures. The problem is not only the small size of the pediatric cancer market, but also the fact that a therapy intended to provide 75 additional years of healthy life for a sick child does not have greater financial value than a therapy intended to add only a few additional months of life for an adult in their 70s. The traditional legislative tools of intellectual property rights and market exclusivity have proven insufficient to address these market failures.

I founded Kids v Cancer the day after Jacob died, with a goal to develop policies and legislation that would correct market failures so that pediatric cancer drug development would proceed at least as quickly as adult cancer drug development. The Creating Hope Act, first passed by the US Congress in 2012 and reauthorized multiple times since, establishes the rare pediatric disease priority review voucher, an incentive designed to correct for the small size of the pediatric cancer market. At the time of Jacob's death, there had only been three drugs developed expressly for children's cancers. The Creating Hope Act has since resulted in dozens of new pediatric therapies and has created over US$2 billion in incentives for pediatric drug development with vouchers trading at around $100 million each — at no cost to the taxpayer.

A further market correction for pediatric drug development, the RACE for Children Act, was passed by the US Congress in 2017. The RACE for Children Act empowers the US Food and Drug Administration (FDA) to require pediatric study plans for targeted cancer therapies in development, thus addressing the lack of pediatric studies of promising adult cancer therapies. In the 12 months following implementation of the RACE for Children Act, 80% of all newly FDA-approved cancer drugs directed at molecular targets substantially relevant to pediatric cancers had pediatric studies planned or in progress, an enormous increase from the pre-RACE for Children Act years.

We are now turning to a third market failure: the value in studying combinations of adult cancer drugs to reach possible cures for kids with cancer, and the hesitancy of companies to undertake such combinations, especially when the drugs are from different companies. The Give Kids a Chance Act authorizes the FDA to require pediatric study plans not only of single adult cancer drugs but also of combinations of adult cancer drugs when there is a strong, scientific rationale.

Advocates for other rare diseases may benefit from our experience as they develop policy reforms based on new scientific approaches, and as they come to experience the political impact of organizing grassroots communities to champion drug development for children. Addressing market failures is likely to require a combination of incentives and requirements; neither alone is sufficient. In designing such incentives and requirements, it is important to consult with academics, advocates, regulators and industry to ensure that the goals are achieved.

This is not the end of the road. There will be an ongoing need for pediatric cancer advocates to reimagine our cancer research and commercialization ecosystem — including increasing financial and infrastructural support for academic studies, repurposing of abandoned therapies, and unique FDA approval pathways for drugs for children with rare diseases.

Society cherishes children's health, but our mechanisms for supporting pediatric cancer drug commercialization are inadequate. I am hopeful that the future will be brighter for children like Jacob — with better treatments that may one day make it possible to be truly cured, to live full and healthy lives. Our children deserve no less. ❐

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