

No Child With Cancer Should Be Left Behind

We need to get targeted drugs to childhood cancer patients more quickly. Here's a proven way to speed up the treatment pipeline.

January 26, 2023 By Lee J. Helman, MD

Every year, more than a thousand children in the United States die of cancer. Thousands more survive but live with side effects of treatments for decades to come. Drugs that could save their lives or reduce lasting side effects are tested only in adults. This is not acceptable. Children may be a small part of the market for cancer drugs, but they are our future and worth investing in.

To make a dent in childhood cancers, we need to get targeted drugs to childhood cancer patients more quickly. We must prioritize research on childhood cancers, regardless that these cases make up less than 1% of the total cancer diagnoses in the United States each year, classified by the National Institutes of Health as “rare cancers.”

Lee J. Helman, MD, the director of the Osteosarcoma Institute and a specialist in pediatric cancer

Courtesy of Lee J. Helman, MD

Earlier this year, President Joe Biden announced a reinvigoration of the Cancer Moonshot initiative to reduce the cancer death rate over the next 25 years and end cancer as we know it. Among the specific targets of the new Cancer Moonshot is the goal of speeding progress against childhood cancers, which affect an estimated 17,000 children a year.

Historically, researchers have had to wait years for drugs developed against adult cancers to start clinical trials for children. This typical sequence puts as many as seven years of waiting between approval of a cancer treatment for adults and approval of the same drug for children. Parents are

forced to watch their children die, out of options, because these drugs are not available to them. Meanwhile, children who survive cancer live with an increased risk for heart disease and other cancers because they were treated with our older and most blunt approaches rather than new targeted, precision drugs.

The Research to Accelerate Cures and Equity (RACE) for Children Act that went into effect in 2020, which requires pharmaceutical companies to more quickly test drugs that might be relevant in pediatric cancers, was a first step. More recently, policymakers introduced the Give Kids a Chance Act, created by the pediatric cancer advocacy group Kids v Cancer. This bill would require pharmaceutical companies to test not only individual cancer therapies but also relevant drug combinations in pediatric populations.

We hope Congress will see to it that the Give Kids a Chance Act is passed into law. But legislation alone will not advance the goals of the Cancer Moonshot. It must be paired with programs that guide pharmaceutical companies to test drugs to actually benefit children rather than check off a policy requirement.

Stand Up To Cancer's robust portfolio in pediatric cancer has already contributed to two Food and Drug Administration approvals: a life-changing, breakthrough immunotherapy called CAR T to treat children with the most difficult blood cancers; and a medication to minimize potential side effects of this new treatment. Most important, this work laid the foundation for a new, successful collaborative approach engaging industry and academia that gets promising drugs into the hands of pediatric clinician-scientists to study as new treatments for childhood cancers, even if the treatments have not yet been formally approved for adults. This approach has been incredibly successful at speeding up the pipeline for childhood cancer drugs and, I believe, can act as a template for how to accelerate childhood cancer research.

Uri Tabori, MD, and his team at the Hospital for Sick Children in Toronto were able to quickly launch new clinical trials in children with "hypermutant" genetic mutations that make them develop multiple cancers at a young age. Earlier this year, Nature Medicine reported that [the immuno-oncology drugs indeed improved survival for these children](#). Another team, led by Kimberly Stegmaier, MD, of the Dana-Farber Cancer Institute, is now testing existing epigenetic drugs—which turn critical cancer genes on and off—in children with a variety of tumor types. Without these close collaborations between Stand Up To Cancer, academia and pharmaceutical companies, these children would not have had access to lifesaving drugs.

Beyond these successful teams—and the work done in collaboration with St. Baldrick's Foundation, a nonprofit that supports childhood cancer research, or made possible with support from donors such as Bristol Myers Squibb—there are thousands of children who still need better treatment options to survive and thrive. Research collaborations like these are examples of what has to play out on a far broader scale. With policies in place that require pharmaceutical companies to test their therapeutics in our youngest patients, now is the time to act to make sure every child with cancer gets a fair shot at survival.

Putting a man on the moon didn't involve just a handful of astronauts with lofty goals. Likewise, to make Biden's Cancer Moonshot a reality for childhood cancers, we need more than great doctors. We need children prioritized in a multipronged, collaborative approach to make deaths and lasting damage from childhood cancers a thing of the past. Despite the polarizing views currently dividing our country on many issues, there's one thing we should all agree on. We cannot allow more of our children to suffer, or be taken from us, due to a cancer diagnosis because we did not make them a priority—our priority. We must leave no child behind.

Lee J. Helman, MD, is the director of the Osteosarcoma Institute and has been studying the biology and care of pediatric patients with sarcomas for over 30 years. He has also trained many investigators in the field of pediatric sarcomas. He spent 30 years at the National Cancer Institute in various leadership positions and recently spent three years at Children's Hospital Los Angeles as the head of research in the Cancer and Blood Disease Institute, where he remains an adjunct professor. He currently focuses on improving outcomes in osteosarcoma.

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