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SPECIAL REPORTS

## The State of Pediatric Cancer Research Funding

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Today, close to 80 percent of children will survive pediatric cancer. However, there has been little to no increase in survival rates of childhood cancer in the last 20 years due to the lack of access to new cancer agents.

Out of the National Cancer Institute's \$4.8 billion dollar budget, only \$173 million, or just over three percent, is allocated for grants that have anything to do with pediatric cancer. Pharmaceutical companies see even less incentive to invest in the research needed to test experimental therapies in children because of the small patient population. Although there are only 12,400 new cases of childhood cancer diagnosed each year nationwide, it is still the leading cause of death by disease in children. Children can't vote, so adults must be their advocates to get more funding and government support to foster research specifically for childhood cancer.



As a pediatric oncologist, researcher and head of The University of Texas MD Anderson Children's Cancer Hospital in Houston, I've experienced firsthand how the lack of federal research dollars stifles momentum in pediatric cancer research and drug development. A drug developed in my lab, MEPACT (miframurtide, L-MTP-PE), is the first therapy in more than 20 years to improve long-term survival for children with metastatic osteosarcoma, a form of bone cancer. Recently the European Union approved MEPACT for use in pediatric patients with osteosarcoma. Even with its demonstrated successes, MEPACT is still sidelined in the approval process for use in newly diagnosed patients by the U.S. Food and Drug Administration (FDA).

Despite these barriers, there's hope in science and pediatric cancer is ripe for advancement. Research for children with cancer - especially for those with solid tumors like sarcomas and brain cancer - lags nearly two decades behind their adult counterparts. There's a lot of work we can do starting with tumor biology - looking at the signaling pathways and other abnormalities that make up the tumor - and working our way toward unlocking the door to the best treatment options to pursue moving forward.

Targeted therapies approved for adults are an area of research that has great potential. For example, we have been able to identify childhood sarcomas that express the same Her2 protein found in certain breast cancers which are being successfully treated with the targeted therapy Herceptin. Similarities in tumor biology suggest that Herceptin might also work in pediatric patients who overexpress the protein. The same holds true for other targeted therapies, like Avastin, that are effective in adults with cancer. These therapies, because they target cancer cells, reducing toxicity to healthy tissue and decreasing long-term side effects, are especially attractive options for children.

To investigate such hypotheses, researchers at the [Children's Cancer Hospital](#) (<http://www.workingmother.com/BestCompanies/childhood-cancer/2010/08/first-person-a-childhood-commitment>) are able to tap into and leverage the extraordinary resources of MD Anderson's adult clinical research program, the largest of its kind in the world. We can correlate our basic knowledge from the biology of certain childhood tumors to adult cancers where approved agents exist. This transfer of knowledge can allow us to test these already approved agents in children quicker.

Because [childhood cancer](#) (<http://www.workingmother.com/BestCompanies/2010/08/september-is>) is considered a rare disease, cooperative trials involving multiple hospitals are almost always needed to accrue enough patients to test a new therapy and get FDA approval. Getting group consensus on the top research priorities or best approach to explore

new therapies is challenging at times. Creative strategies need to be employed in the quest to speed the translation of pediatric research to patient care. We can improve clinical trial design to more efficiently test innovative therapies for rare cancers that affect a limited number of children. Working this way, we can often bypass the need for the large group trials, enabling our patients to reap the therapeutic rewards more quickly.

I am often asked, "[Why do children get cancer? \(http://www.workingmother.com/BestCompanies/2010/08/kids-with-cancer-addies-story\)](http://www.workingmother.com/BestCompanies/2010/08/kids-with-cancer-addies-story)" The answer is: we don't know for sure and without adequate basic, clinical and translational research funding it will remain elusive. Thankfully, with advances in chemotherapy and radiation therapy, so many children with cancer are cured today. New technologies, like proton therapy offered at MD Anderson's Proton Therapy Center, continue to show great promise in treating pediatric patients. However, for each family this disease continues to touch we shouldn't be satisfied with 80 percent of children we are curing today. Rather, we must focus on finding new therapies, new ways to treat the 20 percent of children who are not being cured. That's where we can really make a difference.