

THE CANCER LETTER

Inside information on cancer research and drug development

Kids v Cancer: Laws Need to Catch up to the Science

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By Nancy Goodman

Emma Whitehead was a six-year-old girl battling relapsed leukemia for the third time when her parents were told she had run out of treatments. Her doctors offered one last hope—enrollment in a clinical trial at Children’s Hospital of Philadelphia in a completely new immunotherapy. It was a phase I toxicity trial for both children and adult patients, and few patients on phase I trials are ever cured. But Emma’s family was given the miracle they had prayed for. Five years later, Emma is now a happy, healthy, 11-year-old girl who likes to play piano and soccer. Emma is cured.

It’s a beautiful story and one that you’d love to hear over and over again. However, you won’t. In the United States, drug companies have no obligations to study their promising, possibly curative cancer drugs on kids with cancer.

Kids with cancer are the last, not to first, to get on trials of promising new drugs. And, when drug companies abandon their unapproved cancer drugs, as they do 95 percent of the time for all sorts of reasons, the chance to study those drugs for kids with cancer goes away forever. There is a law in the United States, the Pediatric Research Equity Act, which was designed to address this problem. PREA requires companies developing drugs for adult indications to also develop them for children who suffer from the same indication. However, PREA doesn’t protect kids with cancer because kids don’t get breast cancer or prostate cancer.

The problem is that PREA was written before cancer drugs were developed as targeted therapies with mechanisms of action that might be common between adult cancers, such as breast cancer, and pediatric cancers, such as neuroblastoma. PREA is out of date.

Kids v Cancer has been advocating for the Kids Innovative Drugs Initiative to update and modernize PREA and Best Pharmaceuticals for Children so that the law catches up to the science, and kids with cancer are covered.

Some want to wait at least two years and try to revise PREA and BPCA in the 2017 reauthorization of Prescription Drug User Fee Act, but that is always a highly uncertain process and there is both urgency and opportunity to act now. A large coalition with more than 100 patient advocacy groups and major hospitals is urging Congress to ensure that the 21st Century Cures Act provides cures not just for adults, but for kids with cancer, too.

Last week, kids with cancer achieved a major victory when the European Union's European Medicines Agency revoked existing waivers on drug companies' obligations to study certain drugs in kids, including cancer drugs, when there was a common mechanism of action or a pharmaceutical receptor commonality between the adult and pediatric disease. This decision was a much needed correction to update European law to take into account the shift in cancer research. It's a great victory that the EMA will now require companies to study cancer drugs in kids, but now, more than ever, we need the United States Congress to pass the KIDS Initiative.

The EMA decision will bring more drugs into clinical trials for kids with cancer, but those trials will largely be in Europe, not in the United States. Without the passage of the KIDS Initiative, American kids with cancer still will not have access to trials for promising, potentially life saving unapproved cancer drugs.

Moreover, to comply with these new European regulatory requirements, companies will turn away from the U.S., and U.S. pediatric researchers are concerned that they will have an even more difficult time accessing unapproved drugs for their research or receiving industry funding. It's great that the 21st Century Cures bill recently passed by the House of Representatives provides significant additional funding to NIH, but for pediatric researchers, if they can't get access to unapproved drugs, then they still cannot do their research.

Finally, the EMA program on pediatric development has some problems as well, problems that the U.S. could help fix if the KIDS Initiative were passed into law here in the United States. If the KIDS Initiative were enacted in the U.S., then FDA would have the authority to review and require pediatric cancer studies at the same time as the EMA. The FDA could work with the EMA to ensure that the prioritization of drugs studied for kids with cancer is based on which drugs are most promising for children, not which companies are the quickest in developing drugs for adults.

So, congratulations to the Europeans. They have done right by kids with cancer and other diseases for which targeted therapies are developed. Now, let's pass the KIDS Initiative to bring those benefits home quickly to kids in the United States.

The author is the executive director and founder of Kids v Cancer.

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