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Kids' Cancer Research Bill Proponents Decry Industry Opposition (1)

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- Bill would bring more combination cancer treatments to kids
- Drug industry group says proposed rules are 'premature'

Doctors and cancer patient advocates are pushing back on drug industry calls to delay pediatric cancer legislation that supporters argue is critical to developing more effective treatment options for children.

The bill (H.R. 6972)—the Give Kids a Chance Act—would authorize the Food and Drug Administration to require companies investigating a drug combination for an adult cancer to also launch a pediatric study plan if there are molecular similarities. The measure has bipartisan support in the House and is being pursued as a possible policy rider to this year must-pass FDA user fee legislation, the Prescription Drug User Fee Act, or PDUFA. The law is reauthorized every five years.

Rep. Anna Eshoo (D-Calif.), chair of the House Energy and Commerce Committee's health subcommittee, promised to get the bill across the finish line. She told the bill's sponsor, retiring Rep. G.K. Butterfield (D-N.C.), at a March 17 hearing, "We want to make you glad by getting your legislation through."

Proponents say passage of the bill would mark a win for kids' cancer research, which typically sees fewer studies than adult oncology. But a leading industry group says that while it supports advancing such research, the proposal would burden companies that are still working to comply with a separate measure designed to expand single-drug treatment options.

"It would be premature to make any changes or impose additional requirements," Andrew Powaleny, a spokesman for the Pharmaceutical Research and Manufacturers of America (PhRMA), said.

The bill's backers and pediatric oncologists say they aren't convinced by this argument. "What PhRMA is implying is they hope that we wait until 2027 for the next PDUFA before we ask Congress to consider combination studies—period," said Nancy Goodman, founder and executive director of nonprofit group Kids v Cancer. "That means tens of thousands of kids are going to be sicker and possibly die sooner."

The Give Kids a Chance Act builds on the RACE for Children Act, which Congress passed as part of the FDA's reauthorization (Public Law 115-52) of user fee deals in 2017. The RACE for Children Act allows the FDA to require pediatric study plans for a targeted, single-agent (one drug) adult cancer therapy if it's substantially relevant to a childhood cancer. Patient advocates say the newest bill would help make more combination treatments available, especially for children with particularly aggressive or rare forms of cancer.

"The goal of this act is in the name of this act—literally giving kids a chance," said Rayne Rouce, a pediatric oncologist at Texas Children's Hospital.

"One of the hardest things for me to endure is knowing that there's a potential treatment option, or combination of treatment options that is available and could potentially be beneficial," but "because of legislation or lack thereof, I'm not able to extend or offer that treatment to my pediatric patient," she said.

The FDA said it doesn't comment on proposed or pending legislation.

Combating Drug Resistance

Combination treatments, such as chemotherapy coupled with surgery or radiation, are already common when treating cancers. This approach has shown to be more effective over time, because tumor cells may develop resistance when they are exposed to a single-agent treatment, according to the American Society of Pediatric Hematology/Oncology.

"By complementing the different activities of different drugs, you could get around some of the resistance mechanisms that cancer cells have," said Douglas Hawkins, chair of the Children's Oncology Group, a global research consortium supported by the National Cancer Institute. ▶

Studies into potential combination therapies are ramping up in the adult oncology space, but companies "do not have the financial incentive to do the same for pediatric cancers," said Goodman, who helped craft both the RACE Act and the current bill.

Industry members say the time isn't ripe for new requirements because they're still grappling with how to implement changes from the RACE Act.

The FDA "and biopharmaceutical companies continue to implement these vital provisions aimed at helping pediatric oncology patients," PhRMA's Powaleny said. He added that the findings of an ongoing Government Accountability Office report on the impact of the 2017 changes will likely not be available until August 2023.

'Bankrupt' Opposition

Jim Olson, principal childhood cancer investigator at the Seattle Children's Research Institute, said PhRMA's response is a "pretty bankrupt argument." He argued that the requirements aren't "premature" for "kids with cancer or a parent of a child with cancer," desperate to find a cure.

Doctors and pediatric cancer patient advocates also say the legislation wouldn't impose a blanket requirement on all companies investigating potential drug combinations.

"Companies can still say we don't have enough information, they can get deferrals, they can still say this is really not a relevant combination, and they can get a waiver," Hawkins said.

There is also a large community of pediatric oncology researchers who would be willing and able to work with drugmakers to develop and implement pediatric study plans, he said. "This sort of combination testing is certainly possible outside of a single drug company."

Cartier Esham, the Biotechnology Innovation Organization's chief scientific officer and executive vice president for emerging companies, said at the March 17 health subcommittee hearing that drug companies must balance the desire to enroll children in clinical trials with ensuring they aren't exposed to unknown risks.

The "etiology and biology of cancers that occur in children can be different from those that occur in adults, so immediate extrapolation of efficacy and safety is not always possible," Esham said at the hearing.

Goodman clarified, however, that the Give Kids a Chance Act wouldn't apply to studies of an adult cancer whose makeup is substantially different from a children's cancer. "For the FDA to direct a company studying a combination therapy in adults to undertake that study in children as well, there already has to be a substantial relevance of the molecular targets of the combination drugs to children's cancers."

'Need is Obvious'

Pediatric oncologists and cancer research advocates say the Give Kids a Chance Act is a natural extension of the RACE Act and could be especially beneficial for children with few treatment options, such as those with relapsed leukemias and lymphomas. Some kids may have a specific molecular change or mutation in their cancer that makes it difficult to treat with any single existing therapy, Rouce said.

"The RACE for Children Act was really pivotal in getting cancer drug development to be a priority with the pharmaceutical companies," Hawkins said. The latest bill is "the next step."

Companies are increasingly investing in combination therapy research for adult cancers, and molecular similarities with some forms of children's cancers are a source of hope for pediatric oncologists.

"The need is obvious," Olson said. "We don't have the same resources that pharmaceutical companies have," and "there are parallels between adult cancers and kids cancers, so it's only fair to test them both."

Neuroblastoma, for example, most predominantly impacts children, and has a biomarker, GD2, that is also found in adult brain tumors and other cancers, Rouce said. The Give Kids a Chance Act "opens the door" for possible treatment for children who "may have a completely different cancer than an adult patient, but may have the same molecule that can be targeted," she said.

Goodman argued that while drug manufacturers have been integral to making pediatric cancers less life-threatening, they need to expand research into combinations of drugs that could better treat, and potentially cure, forms of pediatric cancer.

"I'm so grateful for everything the pharmaceutical and biotech industry is doing to develop and commercialize drugs," she said. "I just ask them to do it for kids with cancer as well." ●