

## **For Children With Cancer, Hope for New Treatments**

**A new law requiring pharmaceuticals companies to test cancer drugs on children as well as adults is raising hopes of more therapies**

By Lucette Lagnado

Updated Jan. 15, 2019 9:14 a.m. ET

The health-care industry is preparing for a new law that researchers say will mean more treatments for pediatric cancers, which are the leading cause of death from disease among children.

The legislation, which requires pharmaceuticals companies to test potential cancer drugs on children as well as adults, goes into effect in 2020. Companies already are ramping up and some plan children's drug trials this year.

"It is an incredibly exciting time," said Crystal Mackall, a pediatric-cancer researcher and professor of pediatrics at Stanford University School of Medicine. "We have lots of drug companies who want to speak with us suddenly. Before, we went hat in hand, cajoling."

For years, pharmaceuticals companies balked at trials of children's cancer drugs, which they viewed as risky and unprofitable, some pediatric-cancer doctors said. While adult cancer drugs can be lucrative, drug companies said kids' cancer drugs aren't profitable because of the relatively small market. Of the estimated 1.7 million projected new cases of cancer in the U.S. in 2018, 10,590 involved children age 14 and younger, the American Cancer Society said.  
sym

Dr. Mackall's colleague, Norman Lacayo, a pediatric oncologist at Stanford's Lucile Packard Children's Hospital, said Europe has been much tougher than America on getting drug makers to test therapies on children.

"We were jealous of Europe, where they forced all companies to have pediatric investigational plans for all drugs and we didn't have that," Dr. Lacayo said.

In the U.S., the landscape is changing. This summer at Stanford, Dr. Lacayo will launch a clinical trial of an adult leukemia drug made by Roche on children whose leukemia has relapsed. The trial may help some of his patients, such as 6-year-old Avalynn Wallace, who contracted an aggressive form of leukemia two years ago. Following many chemotherapy rounds, Avalynn kept relapsing and developed infections. She had a stem-cell transplant in September.

“I don’t think there are many options for us,” said her mother, Nicole Wallace. “I think that it is important for drug companies to offer trials for drugs that could cure these horrific diseases children go through.”

More than 80% of children with cancer are cured. However, some pediatric cancers resist treatment and doctors have few courses to recommend. “It is a nightmare for families who have no options. When I see these poor families walk in with their children, you see on their faces their suffering and despair,” Dr. Lacayo said. The trial of Roche’s drug—and similar tests on children elsewhere in the U.S.—will offer hope, he said.

Hubert Caron, who leads the pediatric oncology team at Roche, called the legislation “a game-changer” that will yield more treatments for children with incurable cancers. Roche, of Basel, Switzerland, had already been focusing on kids’ cancers globally, Dr. Caron said, but the U.S. legislation is spurring his company and others to do more, such as the coming trial at Stanford. The drug, Idasanutlin, also will be tested this year on children at Memorial Sloan Kettering Cancer Center in New York. MSK and Stanford are working on the trial with Genentech, Roche’s U.S. pharmaceuticals unit. “We will be seeing major changes in therapies for children in the next five to 10 years,” Dr. Caron predicted.

In 2017, Congress passed the Research to Accelerate Cures and Equity for Children Act, requiring companies to run clinical trials on targeted cancer drugs for kids. Targeted drugs aim to attack genes or proteins specific to cancer cells without harming healthy cells. The RACE for Children Act followed years of advocacy by Nancy Goodman, who lost a child to brain cancer and founded Kids v Cancer, a nonprofit, to push for more research.

Given few cancer drugs for children, pediatric oncologists said, they have been using ones intended for adults on their young patients. “Most of the drugs we use to treat and cure childhood cancer were developed in the 1950s, ’60s and ’70s. It falls off pretty quickly after that,” said Peter Adamson, a pediatric oncologist at the Children’s Hospital of Philadelphia. “There have been cancer drugs developed primarily for children, but the list is quite short.”

Ms. Goodman, whose son, Jacob, was diagnosed in 2007, said she kept hearing about advances against cancer in grown-ups: “I wondered why new treatments were only being developed for adults, not children, with cancer.”

While other legislative efforts tried to get drug companies to do more kids’ research, the firms invariably found loopholes, researchers said; RACE is expected to change that.

Seventeen-year-old Mary Tankersley, who was diagnosed with osteosarcoma, a cancer of the bones, when she was 11, is optimistic about the legislation. She beat osteosarcoma and now is an advocate with the Rally Foundation for Childhood Cancer Research, an Atlanta-based group promoting and funding pediatric-cancer research. “I am not scared of my cancer coming back,” she said. “But if it did—that I could have better treatments: that brings a lot of hope.”

Giselle Saulnier Sholler, director of pediatric-oncology research at Helen DeVos Children’s Hospital in Grand Rapids, Mich., said drug companies have contacted her, offering treatments they consider worthy of clinical trials. Dr. Sholler said she is talking with Amgen, a biotech firm in Thousand Oaks, Calif., about testing a drug to tackle a type of brain cancer in children. “I do think hope is on the horizon,” she said.

Lisa Bollinger, a vice president at Amgen, said her world is changing. She is meeting with pediatric-cancer researchers at academic medical centers to brief them on coming therapies and hear which ones are of interest for testing on children as well as adults.

Some researchers support the legislation, but worry about raising expectations. Dr. Adamson, chairman of the Children's Oncology Group, an international consortium of more than 220 kids' cancer centers, supported by the National Cancer Institute, fears "overpromising patients and families." The law will mean more clinical trials of treatments for kids' cancers, which has a "high value," Dr. Adamson said. But breakthroughs aren't a given. Typically, "we have small steps," he said. "Cancer is a challenging problem."