Fighting kids' cancer, one voucher at a time

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Nancy Goodman's son Jacob was 8½ when he started getting debilitating headaches.

"Jacob ended up being diagnosed on a Sunday afternoon, and Monday we went in to see the neurologist," Goodman said in an interview at her home in Washington, D.C. "Tuesday morning, he had emergency brain surgery."

Jacob had medulloblastoma, a rare brain cancer. After his surgery, Jacob's doctors prescribed a course of chemotherapy drugs.

"He didn't respond, and his doctors knew this chemotherapy regime was not going to work," Goodman said. "So they met and we decided to continue the same regime for the rest of his treatment. Why would his medical team recommend that we do that? It was because there were no other treatments available for this kind of brain cancer."

The protocol they suggested, Goodman said, was 40 years old.

Nancy Goodman

Jacob with his parents Mike Froman and Nancy Goodman, and brother Ben (Spring 2008)

Jacob's situation is not uncommon among kids with cancer, a group that's dwarfed by the number of adults with the disease. Each year in the U.S., about 14,000 kids are diagnosed, compared with 1.6 million adults, according to the American Cancer Society.

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Though they seem rare, kids' cancers have a huge impact; cancer is the second-leading cause of death among children, after accidents. About 1,350 kids under age 15 are expected to die from cancer in 2014, the society says.

Yet very few new drugs are developed specifically for kids' cancers; most drugs used for childhood cancer aren't approved for use in kids, Dr. Ronald DePinho, president of MD Anderson Cancer Center, said at a September meeting on childhood cancer in Washington. As a result, doctors often use drugs that have been approved in adults "off label," meaning in populations other than for whom they've been cleared by regulators, to treat kids.

Lack of access

"We're always opening up the newspaper and we're learning about all of these wonderful advances in science and treatments for cancer," Goodman said. "I was shocked to learn they don't apply to kids. Kids don't get access to these drugs."

Kids' cancers are different from those in adults, which are frequently the result of wear and tear over years of life, said Dr. Stephen Sallan, a pediatric oncologist at Dana-Farber/Boston Children's Cancer and Blood Disorders Center. Yet because of the disparity in market sizes, pediatric cancers are often neglected by drugmakers, he said.

"That 14,000 versus 1.6 million determines a lot of what happens, not because it's right or wrong, but because that's the playing field. It's not level," Sallan said in a telephone interview. "In cancer medicine, virtually all new drugs in the 21st century come to children after they've been tested in adults."

That leads to delays or lack of treatment options that can mean life and death for patients like Jacob. And it brings many patients into the complicated system of applying for compassionate use—access to experimental drugs outside of clinical trials. It's often a move of last resort—and many times, those requests are denied by drugmakers.

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Goodman went through that with Jacob, and after trying several more therapies, he died almost six years ago. He was 10.

Nancy Goodman
Jacob Froman before he contracted cancer.

The next day, Goodman founded a pediatric cancer advocacy group,Kids v. Cancer, with the goal of changing a system that can leave kids waiting years to get access to new drugs—if at all. In 2012, legislation she helped design, called the Creating Hope Act, was signed into law. It awards a voucher to drugmakers that receive approval for a medicine for a rare pediatric disease.

The voucher entitles the company to accelerated review, usually a six-month process, of another medicine by the Food and Drug Administration. That compares with a standard timeline of 10 months. The vouchers are transferable, and in July, drugmakers Regeneron and Sanofi bought the first from BioMarin Pharmaceuticals for $67.5 million, putting a price tag on the incentive Goodman helped create.

An incentive for drugmakers

BioMarin received the voucher when it got approval of the drug Vimizim, for a rare pediatric disease called Morquio A Syndrome.Regeneron and Sanofi said they'll apply it to get a faster review for a cholesterol drug in development, in a class in which they're in a tight race with rival Amgen.

Though Morquio A isn't cancer, the sale showed the program's working, Goodman said. Still, there's more work to be done. Dana-Farber's Sallan points out that most drugs are tested in children only after the medication has been proven to be safe and effective in adults.

"That brings one of these new drugs to a child usually three to five years after it's started downstream in adults, which is just fundamentally, I think, not necessary and probably not right," Sallan said.

Drugmakers may be concerned that a side effect seen in a small study in pediatric cancer could derail an entire program for a much broader adult market, Sallan said. But, he noted, "children are more resilient to the vast majority of new drugs than are adults."

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He suggested companies should test drugs in children alongside adults, establishing safety first in older patients and then extending the medicines sooner to kids. He said regulation could help, by protecting companies from the risk of a child having an adverse event that could affect a drug's entire development program.

Such potential changes are especially important as cancer changes from being defined by its location in the body—lung, breast, prostate—to one based on its underlying genetic characteristics.

Drugmakers say they're looking at the issue. Genentech, owned by Swiss drugmaker Roche, created a pediatric oncology drug development team in 2012, focused on addressing kids' cancers. The group, of 25 people, looks at the science driving cancers and determines whether tumors affecting kids could potentially be targeted by its drugs in development, said Raphael Rousseau, who heads the unit.

"Historically, people have been reluctant (to expose) children to drugs, trying to protect them from research when really the right thing is to protect them through research," Rousseau said in a telephone interview.

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That's a sentiment echoed by Amgen's Dr. Lisa Bollinger, a general pediatrician who spent 12 years overseeing pediatric drug development at the FDA. In a phone interview, she said she joined Amgen in September 2012 to "really look at their entire pipeline and look at opportunities to study their products in the pediatric population."

Developing drugs for kids requires special considerations—about their stage of development, different size and weight, and metabolisms, but also about the concern for longer-term side effects. Rousseau said Genentech has designed a program to follow up with kids for 20 years to monitor longer lasting effects of treatment.

And though progress—through the Creating Hope Act and efforts among industry and government—is being made, Goodman said her work is far from done. She wants to spur a focus on children's cancers not just among existing drugs, but to give drugmakers incentives to pursue drugs for kids even if they don't work for adults.

"When Jacob was diagnosed with cancer, of course I was devastated. But it never occurred to me that there are no drugs available to treat it," Goodman said. "For me it was just a matter of walking down Broadway in New York City, where I lived, and saying, 'How could I possibly be here on this Earth and Jacob isn't?' If the world set up the way it is can't accept Jacob, then I need to change it."