

Genomic screening helps doctors target girl's cancer

October 5 2017, by Helen Adams



Two-year-old Victoria Thompson is one of the first children in the country to have genomic screening as part of the treatment for high-risk neuroblastoma. Credit: Sarah Pack

Victoria Thompson, clutching a bag of salt and vinegar potato chips

while she watches the TV show "Paw Patrol," is a pint-sized pioneer. She doesn't know it, but this two-year-old girl is part of a national precision medicine trial through MUSC Children's Health for children with high-risk neuroblastoma.

Her mother, Jessica Thompson, says Victoria developed a volleyball-size tumor on her kidney last year. Today, after a series of innovative treatments, Victoria's tummy is back to normal toddler size. "This child's a trouper, really strong. You'd hardly even know she's sick," Thompson says.

Victoria's cancer doctor, Jacqueline Kraveka, agrees. "She's done great."

That's great news, considering the high stakes for both Victoria and other children with high-risk neuroblastoma, which has a 60 percent survival rate. About 700 children are diagnosed with the condition in the U.S. every year.

Kraveka serves as vice-chairwoman for the precision medicine trial that Victoria is part of through the [Beat Childhood Cancer Consortium](#).

"What's really exciting about the trial is that we're actually sequencing the tumor, looking at what's altered and then adding a specific drug for that."

The hope is that it will not only fight the cancer now but also reduce the risk of recurrence. "Being a parent, children are the most important thing in your life," Kraveka says. "Children are our future. So being part of something where they have a healthy life and lead a full life is really key."



At one point, the tumor on Victoria's kidney was the size of a volleyball, her mother says. Five rounds of chemotherapy shrank the cancer, and doctors were able to remove it and save her kidney. Credit: Sarah Pack

But it takes more than a good-hearted doctor to beat [childhood cancer](#). It takes research, and Kraveka specializes in that. "We're trying to develop drugs that are more targeted at MUSC Children's Health," she says. "They're drugs that target the genetic makeup of the tumor, thereby improving response and preventing a lot of the side effects that kids get later on."

Kraveka, who has been named a Health Care Hero by the Charleston Regional Business Journal, runs the only translational pediatric cancer research lab in the state. "Translational" means when she sees good

results in her lab at the Medical University of South Carolina, she quickly translates them into treatments for patients. Her work is funded in part by the nonprofit [Chase After a Cure](#), which focuses on cures for hard-to-treat cancers.

Neuroblastoma is definitely one of them. It's the most common solid tumor seen in children under the age of five and the third leading cause of death from cancer in children. "A lot of people have never heard of it," Kraveka says. "The cause is unknown."

Doctors do know that neuroblastoma is a disease of the primitive nervous system that usually shows up in the adrenal gland. Despite its deadly track record, it's not something a pediatrician would typically suspect in a case like Victoria's. Her mom remembers taking her to their doctor in Beaufort, South Carolina, for fevers and a swollen stomach.

"It got to the point where she was in a lot of pain. If we hadn't caught it when we did, she probably wouldn't be here. Follow your instincts," she advises other parents.

Kraveka says the results of the precision medicine trial Victoria is enrolled in, called Peds PLAN—Pediatric Precision Laboratory Advanced Neuroblastoma Therapy—look promising. Kraveka is the principal investigator for the trial in South Carolina.



Jessica and Victoria Thompson live in Beaufort, South Carolina. Jessica encourages other parents to listen to their instincts when they think something is wrong with a child. Credit: Medical University of South Carolina

But that's not the only cutting-edge treatment Victoria is getting at MUSC Children's Health. She's also receiving DFMO, a drug normally used to treat African sleeping sickness that may help keep [high-risk neuroblastoma](#) that's in remission from recurring. A trial with DFMO at the end of standard therapy is also available to patients at MUSC Children's Health.

"It's exciting to be able to offer her something new that gives her a better chance of survival, and we haven't seen an increase in side effects, which is key," Kraveka says.

Doctors think DMFO targets pathways that help neuroblastoma grow and turns them off. An earlier study involving 21 three children with neuroblastoma found that DMFO was safe and may have helped three of them become long-term survivors.

Victoria's mother hopes her little girl, who has been through way more than any two-year-old should have to deal with, is nearing the end of her treatments. "She's had six rounds of chemo. She's had a bone marrow transplant. She's had 20 rounds of radiation," Thompson says.

"She's had a couple of surgeries: one to remove the tumor, which was on her kidney, and it was volleyball size when they found it. Five rounds of chemo shrunk it to softball size, so they were able to get it. They saved her kidney, and she's now doing immunotherapy."

If all goes according to plan, Victoria's treatments should wrap up before Thanksgiving. "You ask the nurses, and they'll tell you, Victoria is such a strong little girl," Thompson says. "She goes through rough stuff during treatment and couple of days later she'll be right back to normal."

Provided by Medical University of South Carolina

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