

METRO

Racing For A Cure

A local medical researcher gets personal about the fight to cure the rare, degenerative genetic disease that afflicts 8-year-old Kirby Wilson.

by Mark Stroh

A small, smiling face looks out over Dr. Robert Yu's medical research lab. At one end of the lab, past rows of shiny black benches piled high with glass tubes, beakers and other more arcane research equipment, three pictures of the face are taped up on one of the tall, glass-fronted cases that rise up from the lab benches.

The happy face belongs to Kirby Wilson. The 8-year-old Chicagoan is smiling in the photo despite the fact she is the victim of a rare genetic disorder called Sanfilippo disease. Her photo hangs in Dr. Yu's lab to remind him and his staff that they are working to save Kirby's life.

Last month Yu, chairman of Virginia Commonwealth University's biochemistry and molecular biophysics department, received a two-year, nearly \$200,000 grant from the Children's Medical Research Foundation Inc. (CMRF), a group set up by Kirby's parents, Brad and Susan, to fund Sanfilippo research. Dr. Yu is using the grant to fund research into treatments and a cure for the disease, which is usually fatal by a child's early teenage years.

Kirby's parents brought her to doctors for testing in April 1995 for what they thought was a learning disability. Blood tests revealed Sanfilippo disease. The disease is one of a series of diseases, known as mucopolysaccharides (MPS), which causes harmful buildups of substances normally produced in the body. Because of enzyme deficiencies, an MPS victim is unable to break down these ordinarily harmless substances, and they accumulate dangerously. In Sanfilippo, the accumulation leads to mental retardation, blindness, stiff joints, behavioral problems and ultimately death.

Susan Wilson says her daughter has lost most of her vocabulary, but she still has a lot of her physical abilities. That's evident over the phone from Wilson's gentle commentary on and instructions to Kirby. Kirby is busy turning on all of the lights in their home, and Wilson adds that Kirby still loves to go horseback riding.

After Kirby's diagnosis, Wilson resolved to do something about the incurable disease. She and her husband found that very few medical researchers were working on a cure for the disease. Researchers need money, they were

told, and more money means more research, so the Wilsons started the CMRF to do just that.

Since getting tax-exempt status in November 1995, the Wilsons have raised \$785,000 to fund Sanfilippo research. By year's end, Wilson says that that figure will break \$1 million.

Wilson heard of Yu from another researcher at Michigan State University. The CMRF awarded Yu an initial grant of \$25,000 in the fall of 1998, and came through in May of this year with a much larger grant, \$88,000 for 1999 and \$93,800 for 2000.

Yu flew to Chicago in February to meet with the Wilsons and the other funded researchers. The parents, and Kirby, made quite an impression on Yu.

"I was really deeply touched by the devotion to their child, and to trying to find a cure for this disease for other kids," Yu says. "It's incredible. I was very deeply moved by this couple, Brad and Sue."

The admiration clearly goes both ways. Yu is soft-spoken, and even though his lab is festooned with pictures of the girl who inspires his race for a cure, he is quick to point out that his research is not just for one girl, but everyone who has the disease. One in 24,000 babies born in the United States has Sanfilippo. "We have a lot of commitment to her, but it's going to benefit a lot of other children," Yu explains.

Wilson says what Yu stops short of saying. She knows he's deeply committed to saving Kirby's life. "He's just a magnificent man," Wilson says. "Not only is his work brilliant, but he has a concern and a compassion, and he realizes more than anyone that we're not only fund-raisers, we're parents."

Wilson says that some researchers only see the scientific side of their work, and don't want to explore the human, personal side. But not Yu.

"He wants to know Kirby," Wilson says. "His desire to help us as parents is what sets him apart. He is so willing to be a part of our personal life with this child. As a human being the man is a magnificent, extraordinary human being."

Yu is working with a team of 16 researchers on several drug treatments that will slow the degenerative effects of Sanfilippo, as well as a cure that involves cell replacement therapy. That

means grafting healthy cells into the brain, in the hope that they will be able to break down the built-up substances that Kirby's genetically deficient cells are unable to. Yu says he hopes to have a treatment he can try out on a human subject in about a year's time, after the necessary clinical trials.

The pace of the research is new for Yu, if not the subject. A veteran researcher who worked at Yale University for 16 years before coming to VCU nearly 12 years ago, Yu was used to taking things slowly and methodically, without deadlines. But Kirby has a deadline Yu can't ignore. Although research is by nature slow, "She can't [wait]." Yu says. "We can all go slow, and must, but she can't, and that makes it harder."

Kirby's mother is comforted nonetheless. Dr. Yu and his compassion just makes me feel that we've got one person out there that is wholeheartedly out to save Kirby with all of his knowledge and expertise, and that's what sets Dr. Yu apart, Wilson says. "The man is just different from anyone we've ever dealt with, and there's a huge amount of comfort there."

Wilson and Yu are both optimistic that the research will be successful, and a year or two will yield an effective treatment for Kirby's disease. In the meantime, while the work proceeds, Wilson gains comfort from her daughter. "What does anyone want from a child?" she asks. "For them to be happy. Kirby is a very happy little girl, and that's all that matters to me. If we can save her, she's got a lot to offer this world."

In his tiny office tucked in a niche at the end of the rows of benches in the lab, Yu speaks more of the medical benefits for every child from the cure he hopes to find than simply saving Kirby. But on a cluttered shelf along one side of his desk, another snapshot of Kirby in a clear plastic frame sits alongside an array of photos of Yu's own son and daughter. Yu speaks proudly of his kids, and then looks for a minute at Kirby's improbably happy grin and says, "I've sort of adopted her, too." **S**