

Promising Autism drug - is out of family's reach - Boy benefited greatly from suramin treatment until UCSD clinical trial ended

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Joe and Rafeef Samo of San Diego knew something was wrong with their younger son when he began regressing socially as a toddler.

He didn't appear to be aware of others at times. He threw tantrums that lasted interminably. He began hurting himself.

After an ordeal in getting an accurate diagnosis for their son, the Samos dedicated themselves to helping him function better despite the disorder that impairs communication and social interaction - autism. They found that intensive therapy brought improvements.

Then came a tiny clinical trial at UC San Diego last year involving just their son and nine other boys. But the results were immense.

Suramin, a century-old drug for sleeping sickness, was repurposed as an experimental treatment for autism. After receiving a single infusion dose of that prescription medication, the Samos' child, whose first name wasn't provided to protect his privacy, achieved major milestones in speech and social abilities.

"The language just literally took off from there. ... With the suramin study and thereafter, he's doing 15- to 20-word utterances. Recently, he's starting to have conversations that center on topics that he's comfortable with, where he knows the answers," Rafeef Samo said.

The Samos have worked on extending those conversations, such as asking their son to name his favorite color or mention things his parents like.

"He's becoming more and more receptive. He's in a great program at school, he's getting support at home," his mother said. "It's still very much challenging, but we're grateful for where he is."

The meltdowns are less severe, she said. Instead of lasting an hour to 90 minutes, they stop after about 15 to 20 minutes. And the boy, 7, has progressed enough that the Samos can teach him coping mechanisms to deal with frustrations.

In general, though, he has lost most of the progress he experienced after receiving the drug. That's because the clinical trial has ended - and with it the access to suramin.

The Samos hope to enroll their son in further clinical trials testing the medication.

UC San Diego intends to launch a larger-scale study involving suramin, but lack of money is holding up the effort, said Dr. Robert Naviaux at the school. Because suramin is off-patent, no drug company has a financial incentive to carry the research forward.

Naviaux depends on charitable donations to advance his team's work, which is expected to cost millions of dollars for the next phase.

If suramin ultimately proves effective in boosting social skills for people with autism, it would become the first drug shown to successfully treat an underlying cause of the disorder.

But even if suramin turns out to be a blockbuster therapy for autism, it can't do its work until the patient gets that all-important diagnosis. While autism spectrum disorder has attracted a huge amount of attention in recent years, the attention often doesn't reach the individual level.

And that's precisely where many families, including the Samos household, struggle to get the crucial help.

It took the Samos an entire year to secure a diagnosis that their younger son has autism - precious lost time because experts resoundingly stress that therapy and support services are most effective when given as early as possible to people with autism. The couple said their then-pediatrician was dismissive of their concerns.

Dayna Hoff knows the Samo family's frustration first-hand. She and her husband, Todd, created the San Diego-based Autism Tree Project Foundation in 2003 after their son Garret was diagnosed with autism. Garret was diagnosed at two years and nine months, and getting that diagnosis took nine months, Dayna Hoff said.

With therapy, Garret, now 17, is doing well.

"It's really disappointing to me to hear that this has happened (to the Samos), but it's not surprising because that's why the foundation even exists," said Hoff, the group's volunteer executive director.

Pediatricians are generally more responsive to symptoms of autism than in years past, but they're at a disadvantage in detecting developmental delays, Hoff said. They simply don't see any particular child enough to gain a comprehensive understanding of their development, she explained.

Rafeef Samo said one reason for the difficulty in getting her younger son diagnosed is that he didn't display the most obvious signs of autism. Until shortly after 2 years old, he appeared to be on a mostly normal development path.

"He had a couple of words here and there (and) he actually met all of his normal milestones. He actually walked earlier than my older son, started saying his first words earlier, crawled earlier, slept through the night.

"But as he got older, we noticed he was getting scared being around my son and his friends. And initially we thought maybe because they were being too rough with him. Whenever new people or new kids would come by, he would kind of get afraid and cling

on to me. When he was about 21/2, we saw that his language was not progressing. It actually slowed down entirely and he was getting very, very hyper."

"And he wouldn't respond to his name. He wouldn't look at us," Samo said. "He did not look like he was present in what he was doing."

In early 2013, the Samos sought answers from their pediatrician, who brushed off their concerns.

"She was telling me things like, 'Oh, your mom just moved in and he's reacting to that,' " Rafeef Samo said. " 'Oh, you got pregnant. Oh, your older son started school. He doesn't have a playmate anymore. You're really reading into it, he's not showing the markers.' "

In mid-2013, the Samos succeeded in getting their son referred to a psychiatrist. That doctor said their son might have attention deficit/hyperactivity disorder, not autism.

As their son's behavior continued to deteriorate, the Samo family went through a crisis.

"By July-August, things were just completely falling apart. It was just spiraling out of control," Rafeef Samo said. "So I started doing my own research online."

She came across a program called First Five San Diego, which provides various free services, including developmental check-ups, for children younger than 5. After their son underwent screening with that program, he was referred to Rady Children's Hospital San Diego.

There, in October 2013, the Samos were told that their son likely has autism. To be sure, they would have to put him through a formal assessment procedure called the Autism Diagnostic Observation Schedule.

"Go back to your insurance," Rafeef Samo said she was told. "Force them to do an assessment. Push for it, push for it, because we really do think your son is showing emerging signs of autism."

On Dec. 12, 2013, the Samos received that definitive diagnosis.

Therapy began early that next year. By then, the family had switched to a different pediatrician and moved into a community served by the Poway Unified School District, which Rafeef Samo praised as a "godsend" for its special-needs program.

"And they really helped him a lot behaviorally. But he was still struggling," she remembered.

As she and her husband continued to search for treatments, they came across the suramin clinical trial led by Naviaux at UC San Diego.

Naviaux's research had led him to conclude that autism was defined by cellular stress that inhibited communication between brain cells. If that stress could be relieved, communication would improve and normal development would resume.

The hypothesis is that ATP, a chemical used for energy in cells, leaks abnormally out of neurons and signals cell damage. The neurons go into survival mode and reduce their communication with other cells. This results in developmental delays for people with autism.

Instead of developing a new medication to treat this cell-damage response, which would have taken many years, Naviaux found a shortcut. He searched through medical data on existing drugs and found that one - suramin - showed the desired treatment properties.

Suramin must be given intravenously, and that process must be performed by doctors knowledgeable in its use to avoid toxicity.

The clinical trial administered suramin to five boys, including the Samos' younger son. Five other boys were given a placebo treatment.

The children treated with suramin showed significant advances in development. These

effects lasted for several weeks after those five boys were given one dose of the drug, and then they mostly dissipated.

For the Samos' son, the improvements came rapidly.

"Immediately after the infusion, a kind of inner cheerfulness started to come out," the parents wrote in a testimonial accompanying publication of the study's results in May.

"When we were walking back to the car, he was holding my hand. He started giggling and looked up at me and said, 'I just don't know why I'm so happy.'

"In big family gatherings, he had a new tolerance when other kids wanted to share his Play-Doh and other special toys. In the past, this would have led to a meltdown. After suramin, that didn't happen and he played much more naturally with the other kids," they wrote.

Today, the Samos continue to help their son with his regimen of therapies.

"We've done 4,000 hours of therapy and (put) many miles on our minivan," Rafeef Samo said. "It's only six years old, and we've put 110,000 miles on it."

She and her husband wait and hope for that chance to give their son suramin again.

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The Samo family enrolled their son in Poway Unified School District, which his mom, Rafeef, called a "godsend" for its special-needs program. Joe Samo of San Diego holds his younger son on his lap at home. The child took part in a clinical trial at UC San Diego last year with nine other boys with autism. Hayne Palmour IV U-T PHOTOS

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