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Parents of Children With Rare Diseases Find Hope in For-Profit Companies

Wealth Matters
By PAUL SULLIVAN DEC. 25, 2015

Karen Aiach, and her daughter, Ornella, who has Sanfilippo syndrome, a rare disease. Ms. Aiach founded a company called Lysogene to focus on genetic therapy. Ed Alcock for The New York Times

Karen Aiach was working as a management consultant when she learned that her first daughter, Ornella, had Sanfilippo syndrome, a rare disease in which a missing enzyme causes toxic substances to build up in the body.

Ornella was 6 months old, and the prognosis was grim: She would develop mentally and physically to between ages 2 and 4, plateau and then lose whatever she had learned. She would become extremely hyperactive and develop sleeping disorders. Most likely she would not live past 15.

Within two years of the diagnosis, Ms. Aiach, who lives in a Paris suburb, had quit her consulting job to learn everything she could about the disease. She hired a neurobiologist to guide her in the world of medical research. And when she learned that few treatments were in the works, she founded a company called Lysogene to focus on genetic therapy.

Instead of raising money and awareness by setting up a nonprofit foundation, a more typical route, she opted to start a for-profit company to seek treatments, if not a cure. Far from common, what Ms. Aiach and other parents like her are trying is to leverage their wealth, contacts and the hope of sophisticated investors to jump-start research into rare diseases.

"My goal was to find a therapeutic approach to that disease," she said.

Such investments can run in the hundreds of millions of dollars; researching and marketing lifesaving drugs can cost billions. And even with their abundance of dedication, many parents start with little knowledge of the science behind the causes of the diseases that plague their children.

But with some rare diseases, where minimal research has been done, a little effort goes a long way.

Nicole Boice, who founded Global Genes, one of the leading rare-disease patient advocacy organizations, said even small investments can have meaningful impacts.

"You can start moving the needle with $3,500," she said. "That leads you to the next $25,000, and then to innovation grants and funding at $100,000. That starts the interest from biotech."

Gradually, parents like Matt Wilsey, a technology entrepreneur, have made headway. First, his family spent the better part of four years trying to figure out what afflicted his daughter, Grace, now 6. Even after her genome was sequenced, the first diagnosis turned out to be wrong. Grace, it finally was determined, was the second person in the world known to have a deficiency in the gene known as NGLY1.

"We went around the country," Mr. Wilsey said. "We were just trying to find one doctor who had seen another patient with these symptoms." After years of efforts, several dozen children have been found to have the same deficiency.

"Our goal is to find a cure," said Mr. Wilsey, who lives in the San Francisco area.

"A lot of people in science dismiss that because cures are rare. But when I say cures, they're not going to be astronauts. They're going to be leading some sort of independent life. They're going to be able to eat without choking. They're going to be able to take a bath without drowning. They're going to be able to communicate, whether with some assistive device or not."

These parents also had a successful model to follow. In 1998, John Crowley left his job at Bristol-Myers Squibb to start a biotechnology company to search for a treatment for Pompe disease, a neuromuscular disorder that two of his children had. Within four years, the company, Novazyme Pharmaceuticals, had devised a treatment that he credits with saving their lives. His story was immortalized in the 2010 film "Extraordinary Measures," starring Harrison Ford. And his company was bought by the pharmaceutical giant Genzyme for $137.5 million in 2001.

Raising money quickly is one big advantage of the for-profit model. Rather than receiving comparatively small donations, the parents behind these companies can potentially raise tens of millions from biotech investors willing to bet on medical trials even without a guarantee of success.

Ilan Ganot, a lawyer who was working in investment banking in New York, learned in 2012 that his second child, Eytani, had Duchenne muscular dystrophy, which affects only 300,000 people worldwide. The prognosis for people who have the disease, considered a severe form of muscular dystrophy, is to need to use a wheelchair by the time they are teenagers. Many die from respiratory or cardiac failure in their 20s.

Mr. Ganot was able to work his banking connections from the start. He raised $17 million in the first year and has since raised an additional $47.5 million.

"The hope is always that we'll be able to become a center of gravity for this one disease," he said. "For me, it was important that the gene therapy was very well-funded."

Mr. Wilsey and his wife, Kristen, started the Grace Wilsey Foundation to raise money to begin research into NGLY1. They contributed their own money but were also successful in raising several million dollars from friends.

But now they plan to start a for-profit company in 2016. Mr. Wilsey was initially hesitant, fearing that it would cannibalize the foundation, but eventually he saw the advantages.

"It provides us more scale and the ability to raise more money," he said. "The company will be focused on many diseases."

Ms. Boice of Global Genes said such persistence and determination could attack some of the 7,000 known rare diseases that affect about 350 million people worldwide. Only 30 percent of the diseases have any coordinated effort to raise money or encourage research, she said, and 500 of them have treatments.

"I'm not saying it's a smooth ride," Ms. Boice said. "You're investing in animal models, aggregating data and flying around the world to meet experts. And some people have been at this game a long time, if you think about how much money has been raised for cystic fibrosis and muscular dystrophy, and we don't have a cure."

Mr. Crowley himself emphasized that success is not always repeatable. When he began, he knew little about the science behind what he was doing, and he was not wealthy. And though he sold his company, he cautions others seeking cures for the sake of financial reward.

"A lot of times people will call and say, 'We read about what you did and we want to start a company,' " he said. "That's not always the right answer. There are a lot more companies willing to work on rare diseases than there were two decades ago. I urge people to take advantage of those entrepreneurs."

Mr. Ganot, who said his company would start trials in 2017, acknowledged that various factors would determine if the treatment was successful. "The human body isn't simple, and this is complicated," he said. "We got to get lucky, too."

Yet Mr. Crowley understands what drives these parents. "I wouldn't have wanted to have looked back years later and said, 'God, I wish I could have done something else.'"

And even small breakthroughs can buy patients time. Ornella Aiach's disease had become quite advanced by the time she turned 6 and she became the first patient to receive the gene replacement treatment that Lysogene had developed.

"She was extremely hyperactive when she was dosed with the product," Ms. Aiach said. "Afterwards, she started to do much better."

After financing the research for years from their savings and contributions from friends, Ms. Aiach recently raised $18.6 million from outside investors and hopes the money will allow her to broaden the trials on the treatment her daughter receives and make more progress.

Ms. Aiach realizes a cure for her daughter may not be possible, but the treatment could offer far greater hope for others who are treated earlier in the progression of the disease. The gene therapy may also have applications for people with Parkinson's disease.

The treatment has, in effect, slowed the ravages of Sanfilippo syndrome. Her daughter is now 10 and can walk with assistance. She cannot speak, but she is not hyperactive and can sleep through the night.

"Doctors had told us that it would take 15 or 20 years to develop a treatment, and it would be too late," Ms. Aiach said. "The way drugs and treatments are developed now is changing a lot. Parents are playing a large role."

Correction: December 25, 2015

An earlier version of this article described incorrectly John Crowley's children who had Pompe disease, a neuromuscular disorder, and who inspired him to start the biotechnology company Novazyme Pharmaceuticals. The two children are not twins.

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