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Coaxing Cancer Researchers To Take Your Money

Sometimes Even Large Sums
Can't Attract Scientists
To Investigate Rare Diseases

By **AMY DOCKSER MARCUS**
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In 2004, Marnie Kaufman was diagnosed with a rare, little-understood cancer. There was no effective chemotherapy. Her husband Jeffrey's first thoughts turned to how to jump-start research. "Jeff wanted to raise money and give it to anyone walking down the hall of the hospital," Mrs. Kaufman recounts.



Marnie and Jeffrey Kaufman took a proactive approach with their Adenoid Cystic Carcinoma Research Foundation.

But after meeting with David Sidransky, director of head-and-neck-cancer research at Johns Hopkins University, and asking him to work on the organization they started, Dr. Sidransky told them to think about an issue they never anticipated: What if no one wanted their cash?

Budgets are tight at the National Institutes of Health, labs are scrambling to find funding, and many private foundations and pharmaceutical companies don't invest in research for rare cancers. But for many patient advocates, there is a further obstacle. Sometimes they raise money and get no takers.

A friend of the Kaufmans who started his own rare-cancer advocacy group gave the phenomenon a name: the doom loop. Some researchers -- tired of getting turned down for grants and fearing there will be no funding to sustain long-term research -- don't bother applying even when money becomes available.

To avoid that trap, the Kaufmans tried something different. They raised more than \$700,000 for Mrs. Kaufman's disease, a salivary-gland tumor called adenoid cystic carcinoma, or ACC, and then, instead of sending out a call for proposals and waiting for responses that might never arrive, they took matters into their own hands. Working with a small, four-person scientific board, they came up with their own list of projects they wanted done. They did research into who had expertise in those areas. Then they went out and essentially hired these experts to work on specific projects.

Q&A

The Kaufmans used a combination of approaches to lure experts who might never have sought grant money

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¹ • A Q&A with Dr. Isis Mikhail²

on their own. Sometimes they targeted researchers doing work for other cancers and persuaded them to apply that work toward ACC. They offered not just money, but other support, such as help collecting data or materials. And they sped up the often monthslong

traditional grant process by going straight to the researchers with a plan in place. They either signed business contracts or gave out grants, but they always listed specific goals and strict time frames. Generally, the Kaufmans made it easy to say yes.

Getting Results

The approach is beginning to speed up results for ACC, which affects fewer than 1,000 people a year. There are now new mouse models for the disease -- lab mice that carry the tumor -- that will be used to test more drugs. And a lab is conducting gene sequencing on tumor samples, among other projects.

It hasn't been easy. The effort requires a level of resources and commitment that not everyone can muster. The couple, who have no medical background themselves, had to become extremely knowledgeable about Mrs. Kaufman's disease and work closely with their scientific advisers. Some researchers are also wary of starting a project on a rare cancer like ACC, for fear funding down the road could dry up. But the Kaufmans see their work on ACC as a possible blueprint for other small, overlooked diseases. "Once a handful of us go through it, we can help others do it," Mr. Kaufman says.

Other groups are getting more aggressive also. The Cystic Fibrosis Foundation used to spend the majority of its research funds on investigator-initiated ideas. Last year, only 34% of the money was used that way, according to a foundation spokeswoman. Now the foundation is more likely to pursue companies it wants to do drug trials. Organizations such as the Juvenile Diabetes Research Foundation and the Michael J. Fox Foundation have also escaped the doom loop by putting up millions of dollars to get pharmaceutical companies to test drugs.

When Mrs. Kaufman, now 41, was diagnosed, her youngest boy, Max, was only one. The Kaufmans, who live in Needham, Mass., have three other children: Ben, 11, Skyler, 9, and Toby, 7. Because there is no known chemotherapy that is effective for ACC, Mrs. Kaufman had surgery to remove her tumor and then seven weeks of daily radiation. "The huge Frankenstein scar on the side of my neck seems to be the focal point for the kids right now," Mrs. Kaufman wrote in an email that she sent out to friends and family after her surgery. "Toby can't look at me unless he looks at the other side."

She finished the last radiation treatment in October 2004, the same day "the Red Sox whupped the Yankees and made it to the World Series. ... Coincidence? I don't think so," she wrote.

In that email, she struggled to explain to them -- and to herself, she says -- the kind of uncertainty that shadowed her even after treatment. Unlike many cancers, ACC is typically slow growing. But it is insidious and relentless, typically invading nerves and spreading throughout the body. While five-year survival rates are pretty good, at 15 years, the survival rate drops to only 40%. Many people with ACC undergo numerous surgeries or live with painful tumors that spread to other parts of the body, such as the lungs and bones.

"The good news is," she added in that email, "it is reasonable to hope that if it ever came back ... it may not be for many years, at which time there could be a better cure."

That was the driving idea behind setting up the Kaufmans' Adenoid Cystic Carcinoma Research Foundation. In the nine months after that last email, the couple met with researchers and other patient advocates trying to figure out the fastest way to get the work done. They joined an online patient-support group started by Prudence Jackson, who founded the Adenoid Cystic Carcinoma Organization International in 2003. That organization raised money for research, including at least 12 \$30,000 grants given out with the National Organization for Rare Disorders. These grants resulted in, among other things, ACC gene research.

Waiting for Proposals

But Mary Jean Sawey, a member of NORD's medical advisory board who reviewed ACC proposals, said that the drawback in waiting passively for proposals is that "some are very innovative and work out great and others are just fishing expeditions."

Dr. Sawey says the Kaufmans' approach has its own difficulties. "When researchers come to you with an idea, it's because they want to do it," she said. "This way, you have to convince them to do what you want to do, and you end up having to be a manager."

Mrs. Kaufman had spent the seven years prior to her diagnosis raising her children. But for many years, she worked as a corporate-foundation fund-raiser for a major hospital in Boston. After starting the ACC organization, she used those skills to tap friends and family. She sent out holiday cards with personal messages explaining their efforts to fight ACC. She mailed ice cream to researchers who met with them. Around the country, supporters ran golf tournaments, 5K races, silent auctions and other events, quickly netting more than \$700,000.

Finding the Right People

"Raising the money wasn't the hard part," Mrs. Kaufman says. "Finding out that raising the money wouldn't be enough, that I was going to also have to find the right people to give it to, was really hard to accept."

But fatalism can keep researchers from coming forward on their own. For instance, Barry Ziober, an assistant professor at the University of Pennsylvania, got a \$30,000 grant from NORD two years ago to do a project on ACC. But after the money ran out, he felt he still didn't have enough data to convince the NIH to fund him -- so he didn't even apply. And he felt it would be futile to go back to NORD because he had already received funding, and "organizations like to spread their money to different people."

But officials at NORD say this is not the case. "Dr. Ziober would have exactly the same chance if he applied again for another grant," says Stefanie Putkowski, research program administrator at NORD, adding: "We have doctors who get repeat grants."

The Kaufmans have been in touch with Dr. Ziober about a possible project, but he says he is currently focusing on work that already has funding. So, Dr. Ziober says, his ACC work "is sitting in a folder on my desk."

After researching where to direct their group's money, the Kaufmans decided they needed more mouse models. But very few existed. "There is no glory for researchers in growing ACC tumors in mice," says Mr. Kaufman.

At Dr. Sidransky's suggestion, they reached out to the Institute for Drug Development in San

Antonio, whose researchers develop new treatments for cancer. The Kaufmans offered IDD not just money, but also help in gathering tumor samples. Quickly, they signed a \$70,000-plus contract for IDD to expand the number of mouse models and test 14 different compounds to see if one or a combination of them slows or stops the disease. The information discovered will belong to the organization, which can then pursue further drug development.

Earlier this year, the Kaufmans' organization gave a grant to the Wellcome Trust Sanger Institute in Hinxton, England, to do gene sequencing on ACC tumor samples to search for potential new drug targets. The Kaufmans drew up a list of labs that did such work, requested proposals specific to ACC and compared prices. Rather than wait for a regularly scheduled board meeting to review the proposals, Mr. Kaufman circulated them via email, set up a teleconference the same week, and negotiated the deal with Sanger.

Sample Collection

One of the researchers whose help they sought is Adel K. El-Naggar, director of head-and-neck pathology at M.D. Anderson Cancer Center in Houston. He had been collecting samples of tumors from a number of cancers, including ACC. With encouragement and money from the Kaufmans' group, Dr. El-Naggar has hired people to harvest, annotate and disperse tissue from ACC tumors and collect clinical and follow-up information from the patients. He says he never would have considered requesting money to do such work. For such projects, says Dr. El-Naggar, there has never been "a funding mechanism" for sustained research.

Mrs. Kaufman says she is cancer-free for now, though she is monitored frequently, including magnetic resonance imaging of her head and neck, an annual chest and abdominal CT scan, and her doctor's manual exam of her neck glands to make sure there are no new tumors. In the past six months, she had to undergo additional tests due to pain in her back that turned out to be caused by benign cysts, and a growth on her head that also was not cancerous.

"This is the kind of cancer that can come back anywhere," she says.

Mr. Kaufman decided in March to take a one-year leave-of-absence from his job as an investment manager at Putnam Investments in Boston. He went to work full-time, without pay, at the organization because it has been challenging to understand the science and coordinate the different projects. "Even the best-intentioned researchers have huge demands on their time," he says.

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