The CEO Who Saved a Life and Lost His Job

By Caroline Chen January 22, 2015

Photographer: Jeremy M. Lange for Bloomberg Businessweek

Former Chimerix CEO Moch

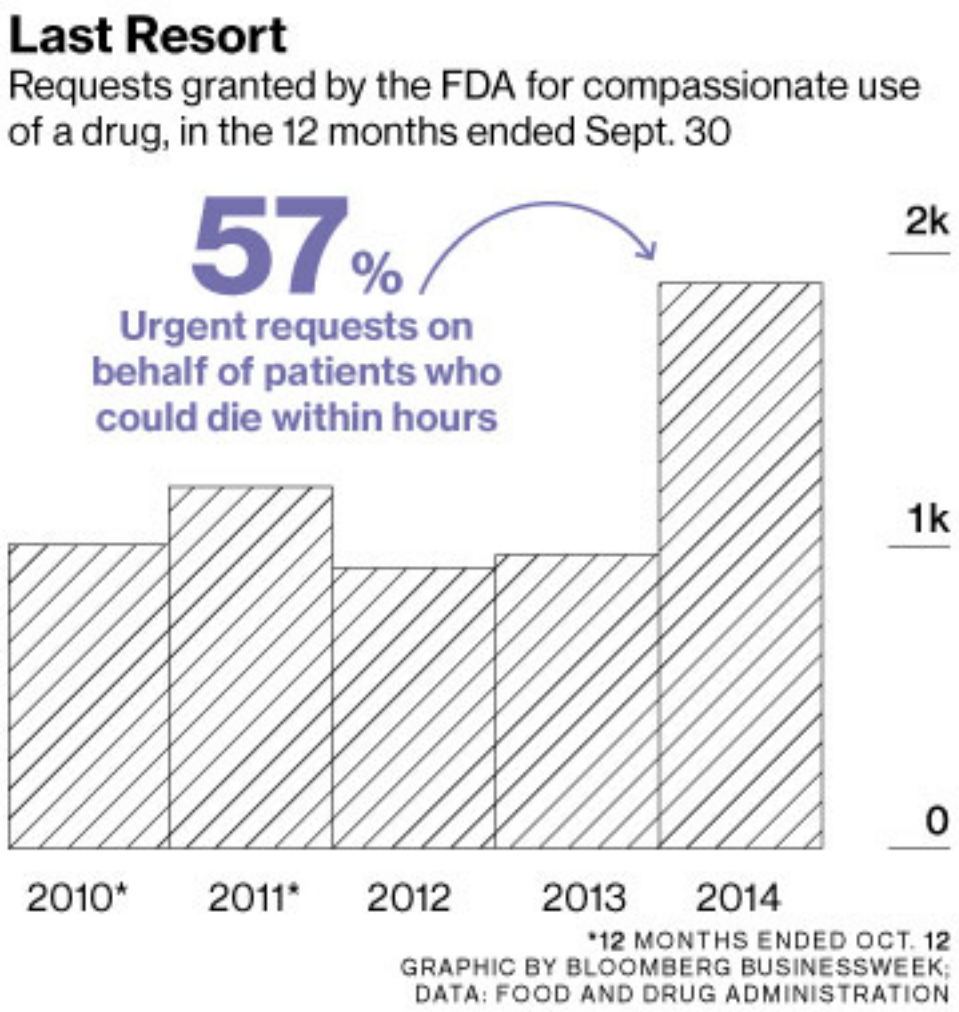
In early 2014, 7-year-old Josh Hardy was dying. He'd been battling kidney cancer since he was a baby and survived 10 intensive chemotherapy treatments, which severely weakened his immune system. Soon after

undergoing a bone marrow stem cell transplant last January, he developed a life-threatening respiratory virus.

Chimerix, a small biotechnology company in Durham, N.C., had an experimental, unapproved drug in development that might help, Josh's doctor at St. Jude Children's Research Hospital told the boy's family. They twice asked Chimerix to provide Josh the drug and were refused both times. "Please help us save our son," his mother, Aimee, wrote on her Facebook page on March 6. "If anyone with influence can help us convince the Chimerix Inc to release the drug for compassionate care for our son, we would be forever grateful."

Within several days, a #SaveJosh hashtag was trending on Twitter. A Facebook page set up for Josh's cause registered 27,000 "likes" in one week. Social media posts supporting the family's effort to get the drug were directed at Chimerix and its chief executive officer, Kenneth Moch, who also received thousands of e-mails and phone calls. If Josh dies, asked one tweet, "how will Moch live w/himself?" Another said that Moch's decision to withhold the drug carried "a HEAVY Karmic price!" Yet another called Chimerix executives "cowards … Ken Moch, I can only hope this doesn't happen to your child." And Moch was the target of death threats that the FBI deemed credible, so he and his wife moved out of their home for several days.

Under intense pressure, the company worked out a plan with the Food and Drug Administration to add a clinical trial for the drug, brincidofovir, and to enroll Josh as its first patient. "It was relatively an easier campaign because we had a very tangible goal," says William Burns, Josh's uncle, who first used the hashtag and set up the "Save Josh" Facebook page. "We had an immediate need, not like, 'Give more money to cure cancer 10 years from now.' "'



While the question of whether to give Josh the drug seemed like a no-brainer to his family and supporters, it presented Chimerix with tricky issues to sort out. Compassionate use—formally known as expanded access—allows experimental drugs that haven't been approved by the FDA to be given to patients in life-or-death situations who have no other treatment options. While the FDA is required to sign off on compassionate-use cases—it usually approves about 1,000 a year—the decision about whether to give out an unapproved drug rests with drugmakers. A patient's physician typically requests a drug from the company developing it. The company weighs several factors, including the most potentially tragic—that the drug might hasten or cause the death of a patient. Such a death can complicate a company's efforts to secure FDA approval and to ultimately make the drug widely available.

For a small biotech like Chimerix, which had no product on the market and no source of revenue, handing out experimental drugs, usually for free, can be especially costly. Compassionate use also can delay the FDA approval process, because drugs and other resources are diverted to patients who aren't enrolled in formal trials, which collect data necessary for gaining regulatory approval.

"Everyone asks this question," Moch says. " 'If it were my child, would I do what the Hardys did?' Absolutely, yes. " But that wasn't his role, he says. "As the CEO, I have to think not just about the individual, but the many."

On April 9, three weeks after Josh got into the brincidofovir trial, Moch stepped down as CEO. The company says he resigned. Moch says he was replaced and can't comment in detail about his departure other than to say he would have "loved to continue to be CEO, but the board felt otherwise." Chimerix declined to comment beyond its press release announcing Moch's departure and his replacement. "We are now entering a critical stage of the clinical development of brincidofovir," the release said. "Our goal is to complete the requirements for regulatory approval of brincidofovir as rapidly as possible."

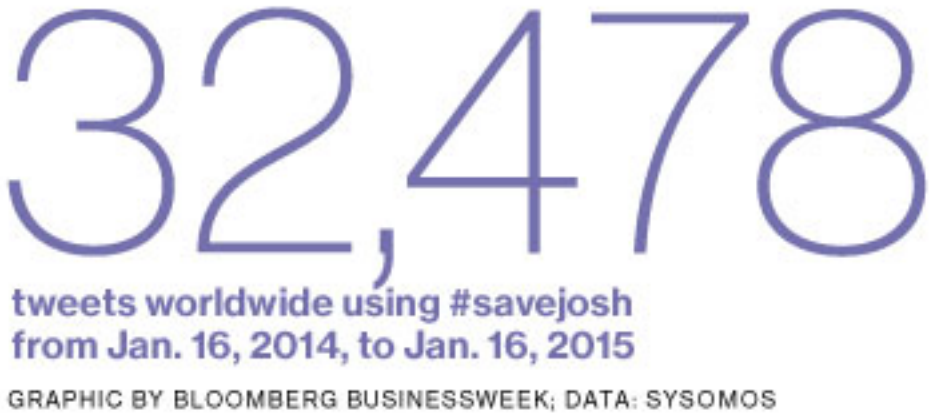
Moch, 60, joined Chimerix as CEO in 2010. He studied biochemistry as an undergraduate at Princeton and had worked in the life sciences industry since getting his MBA from Stanford in 1980. In 1982 he co-founded Liposome, a developer of treatments for cancer, including breast cancer; drugmaker Elan acquired Liposome in 2000. Moch held various executive-level positions with medical companies before joining Chimerix.

Last year, when Chimerix was approached for help by the Hardy family, the company had 55 employees, no approved drugs, and was relying on funds from investors. It was in the final phase of testing brincidofovir for FDA approval to market the drug to prevent cytomegalovirus in bone marrow stem cell transplant patients. Chimerix also had tested brincidofovir as a preventive therapy for adenovirus, the virus afflicting Josh, in an earlier stage of the trial. At the end of March 2014, the company had an

accumulated deficit of $173 million since its founding in 2000, according to regulatory filings.

Chimerix had previously given brincidofovir to 430 patients for compassionate use in a program funded in part by the government. When funding ran out in 2012, Chimerix closed the program to focus on getting the drug through the FDA, Moch says. Allocating any available doses to Josh could have slowed that process, he says. “It’s horribly conflicting,” he says of the dilemma he faced in March. “You go into this business to saves lives.”

Some patient advocates insist that companies have a moral obligation to make their drugs available to desperately ill patients. “Here’s a kid where it’s life or death, hanging on to Ken Moch’s decision,” says Nancy Goodman, executive director of Kids v Cancer, a nonprofit advocacy group for children with cancer which helped publicize the Hardy family’s campaign. “It’s profoundly unethical for him not to give the drug.”



Kevin Donovan, director of the Center for Clinical Bioethics at the Georgetown University Medical Center, says the conflict between companies and patients lies in a clash of responsibilities. “The company’s obligation is the greatest good for the greatest number,” he says. “If it’s your loved one, if it’s you, if you’re a patient, your moral obligation is to the welfare of that individual.” Both approaches are legitimate, he says.

These complexities get lost in a social media maelstrom, says Darshak Sanghavi, an associate professor at the University of Massachusetts Medical School who's conducted research on compassionate-use issues. "I think that whenever you have 140 characters to describe a complicated medical decision, it's going to be oversimplified— 'there's a dying child, why won't the drug company give the drug?' " Sanghavi says. "It's so easily amplified, and any nuance, even if it was present early on, rapidly gets rubbed out," he says.

James Greenwood, CEO of the Biotechnology Industry Organization (BIO), says social media's role in the Hardy case unnerved the industry. "When you add social media and start tweeting all over the place, then reason flies out of the window," says Greenwood. "Social media creates opinion storms, we all know that."

Moch stresses that in the end, Josh's brincidofovir treatment was not compassionate use. As part of a new clinical trial, his response to the drug was included in data Chimerix was gathering in its push for FDA approval. The trial tested brincidofovir as a treatment for adenovirus, not just a preventive therapy. Barring any delays, the FDA could approve the drug by the end of 2016.

Moch's point is one that matters deeply to small biotech companies trying to get their first drug to market. One example, says BIO's Greenwood, is CytRx, a biotech whose trials for its experimental cancer drug aldoxorubicin were temporarily placed on hold on Nov. 18 after the death of a patient who received the medication under compassionate use. The potential for the FDA to stop a trial because of a patient death or some adverse event outside the scope of a trial, for which patients are carefully screened, could further deter biotechs from giving out their drugs, Greenwood says.

BIO is working with its member companies, particularly small ones, to prepare for campaigns like the one the Hardy family waged against Chimerix and Moch. The group suggests that members make it clear how a

compassionate-use program will operate and develop a crisis management plan.

About a week after receiving brincidofovir, Josh's mother reported on her blog that his condition had improved dramatically; he was discharged from the hospital in early April. Moch says he's in active discussions to take a new CEO job. He's spent the past nine months meeting regularly with chief executives of other small companies about compassionate use issues and consulting for a working group of ethicists at New York University's Langone Medical Center that's studying the challenges of compassionate use.

"For the biotech community, what happened to Moch was a shock," says Greenwood. "Within the BIO board and in our community, Ken Moch is a very loved person. People were hurt to see what happened to him, and they worried that this could be me."

The bottom line: *Biotechs have watched closely the experience of Chimerix, the target of a social media campaign.*