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MEDICAL DISPATCH

MOTHER COURAGE

A family tragedy and a scientific crusade.

BY JOHN COLAPINTO

In the early nineteen-eighties, Pat Furlong noticed that her infant son, Patrick, was "floppy." He would slip through her hands when she attempted to lift him from under his armpits. "You'd stand him up," Furlong says, "and he'd just kind of blob down." Her two daughters, then seven and five, were developing normally, but she began to look with concern at the other son, Christopher, who was two years older than Patrick. He, too, had been delayed in his motor skills; he had walked a little later than other kids, and now, at three years old, he could not turn a somersault. Furlong took the boys to doctors all over their home town, Middletown, Ohio. She was told that there was nothing wrong with them. Both boys were smiling and engaging, clearly normal in their mental functioning. But Furlong, a health educator and a former nurse, remained worried. "For me, it was a feeling in my stomach that something was wrong," she says. She complained to her husband, Tom, a family-practice physician, that other children in the neighborhood ran and jumped while Chris and Patrick coloring. Tom dismissed her fears, saying that the other kids were simply unxly.

As a nurse, Furlong had spent years examining illness. She grew up in Cincinnati, the youngest child of first-generation German-American parents, and in 1969 she earned a nursing degree from Mount St. Joseph's, a local college. "I'm fascinated by medicine—what we can do and can't do," she told me. In graduate school, at Ohio State, she ran the intensive-care unit at the campus hospital. But her experience caring for adult patients was of no help in trying to understand what was wrong with the boys. "If an adult was sick, I could go through it systematically and say, 'What are we looking at?'" she said. "But with children I didn't have those tools."

When Chris was six, he suffered an unexplained injury to his calf. "He was riding his Big Wheel tricycle," Furlong told me. "Pointing his toe and flexing, he said all of a sudden that something hurt. And he started to cry. It was weird, because what could he have done? His calf muscle started to really swell." Enlarged calves are leading diagnostic indicator of Duchenne muscular dystrophy, a rapid, fatal muscle-wasting disease that affects males almost exclusively. As the muscles die, they are replaced by scar tissue that looks, to the uninitiated, like increased muscle mass. Furlong had noted the unusual bulk of her sons' calves, but had taken it as an indication that the boys would grow up to be big, like their father, who had played football for Notre Dame.

Tom seemed mystified by Christopher's injury. But the next morning Furlong saw him looking at Chris with tears in his eyes. "I said, 'What are you looking at?'" Furlong recalls. "And he said, 'I don't know.'" Later, Tom admitted that the possibility of Duchenne had crossed his mind. "But it's not a diagnosis you want to make," Pat says. "And there was no family history of the disease."

Still hoping that the injury was a sprain or a muscle tear, Furlong made an appointment with an orthopedic surgeon in Middletown. The doctor saw Chris's calves and his distinctive gait—he lifted his shoulders and pushed his pelvis forward, to help swing his weakened legs—and knew immediately that the boy had Duchenne. "He didn't even have to do any tests," Furlong says. "He said, 'What does Patrick look like?' And I said, 'Well, he's the same.'"

Furlong was referred to Cincinnati Children's Hospital, where the boys underwent three days of testing. A neurologist there confirmed the diagnosis of Duchenne, and laid out a dire prognosis. The first signs of the disease usually
appear in early childhood and are followed by rapid, progressive loss of muscle strength that leads sufferers in a wheelchair by their early teens and eventually renders them completely immobile. Victims typically die from cardiac or respiratory failure, often before the age of twenty. Since 1861, when the French neurologist Guillaume Duc-

chenne first described the disease, no drug had been developed that affected its inexorable course.

Furlong recalls that the neurologist told her, “There is no hope and no help—just take them home and love them. They’re going to die.” Duchenne is a genetic condition, coded on the mother’s X chromosome, and is usually inherited. The doctor upbraided her, she says, for having had a second boy. “You should have known about this,” she recalls him telling her. “This is a familial disease, it’s genetic, you have it in your family.” I said, ‘I don’t.’” (She later learned that she was among the one-third of cases in which the mutation appears spontaneously.) The doctor insisted, “You could have prevented

Furlong was determined to fight the disease, but Tom, as a physician, tended to accept the prevailing medical view that nothing could be done. He began to prepare for when Christopher and Patrick could no longer walk, building a wheelchair-accessible addition to their house and installing a small elevator. Furlong was furious: “I wasn’t sure where I am now, able to stand back and say, ‘He was crushed in his own way, and he was trying to survive.’” Tom withdrew from the family, shutting himself in his home office in the evenings and refusing to discuss the diagnosis with anyone. The two grew apart, as many Duchenne parents do.

As a nurse, Furlong understood that her sons would probably die of Du-

hundred and fifty thousand sufferers worldwide, between ten and twenty thousand of them in the United States. Duchenne is classified by the World Health Organization as a rare disease, and scientists often see little incentive to specialize in such diseases, because few research dollars are available. Thus, the burden of stimulating new research often falls on patients or their parents.

Furlong, at sixty-four, is a tall, attractive woman with a dramatic presentation—bright-red lipstick, ruffled black tops, artfully mussed brown hair—and a manner that blends unyielding resolve with self-deprecating humor and a certain sadness. She has been singularly effective as a parent activist, not only in spurring research into a
cure for the disease but in working with doctors and drug companies to improve care. In 2001, she helped lobby Congress to pass legislation that has allocated more than four hundred million dollars for research into muscular dystrophy, of which more than a hundred and sixty million has gone to Duchenne research—for outstripping the research money raised for Duchenne by the Muscular Dystrophy Association.

Dr. Steve Croft, the director of the Office of Rare Diseases Research, at the National Institutes of Health, told me recently that much of the current clinical research into the disease is the result of Furlong’s efforts: “She has been the major mover with Duchenne muscular dystrophy—around the world.”

Less than a week after her sons received their diagnosis, in June, 1984, Furlong went to the bank and, without her husband’s knowledge, borrowed a hundred thousand dollars. “The president of the bank said, ‘Does Tom know?’ Furlong recalled. ‘I said, ‘Of course—would I be here if he didn’t?’’” She forged his signature as co-signer on the loan. Tom found out and “went wild,” she says. When she told him that she was going to use the money to save their sons, he told her that it was a futile effort, but he did not force her to return the money. Furlong says he knew that “if he messed with me he would lose me.”

Furlong began travelling to medical centers around the United States and Europe. “I wanted to understand the landscape—who the players were, what they thought, how they thought about Duchenne, what priorities, what plans,” she says. She found that very little was being done, and that few doctors were willing to talk to her. “Most researchers and physicians will do anything to avoid meeting with distraught mothers,” she says. To get past secretaries, she would impersonate a doctor on the phone, then arrive at meetings dressed for the part. “I would wear something very professional and the highest heels possible,” she said. She added, “Of course, I’d start crying the minute I sat down with them—so they knew.”

Not long into her research, she read a paper by Dr. Charles Bonsett, a neurologist in Indiana, who reported promising early results from adenoxygucinate acid. She flew to Indiana to meet him. No clinical trials for Duchenne existed, but Furlong persuaded Bonsett to launch one using the compound. She helped fund it with the money she had borrowed from the bank, and made sure that her sons were included. They took the drug—which was administered by a shunt surgically implanted in their abdomens—for years, but their slide continued. At eight, Christopher lost the ability to walk; the same year, Patrick, whose Duchenne was more aggressive, lost the use of his legs, too. Subsequently, they enrolled in a treatment, overseen by a doctor in Memphis, that involved the transplantation of immature muscle cells, called myoblasts. It was not more successful, and Furlong withdrew them from the trial in 1993.

By then, doctors had discovered that steroids can slow muscle wasting in Duchenne boys, extending life into the twenties and sometimes beyond. (The drugs have serious side effects, including severe mood alteration, weight gain, stunted growth, suppressed puberty, and increased bone fragility.) Clinical trials of steroids for Duchenne were in progress, and Furlong tried to get her sons included. But, when she was told that a placebo group would not be given the treatment, she withdrew them, rather than take the risk that one of them would not receive the drug. Instead, she asked Tom to write a prescription for steroids. He refused, saying that they could not perform unregulated drug experiments on their children. Furlong, incensed, forged his handwriting on a prescription, but finally decided not to fill it. “I did not want to be culpable,” she said.

At the ages of fifteen and thirteen, Christopher and Patrick were fully paralyzed, except for their organs of speech and their fingers. They moved around in motorized wheelchairs using joysticks, and relied on family members and a few paid helpers to lift them onto the toilet, or from wheelchair to bed. Unable to raise their arms, they ate with their elbows propped on high drafting tables that their father rigged for them. Despite these difficulties, Christopher regularly ranked at the top of his class in school; Patrick concentrated on friends and dreamed of success as a gambler. When the myoblast treatment failed, they were crestfallen. “Chris asked, ‘Have you given up on us?’” Furlong recalls. She assured him that she had not, and shortly afterward, in 1994, she decided to start a patient-advocacy group.

“By this time, I was well acquainted with the small group of researchers in the field,” Furlong says. “I thought it was time to call them together with the intention of funding research. To raise significant dollars, we needed to form a nonprofit.” She got in touch with Duchenne parents whom she had met through clinical trials, and incorporated the organization under the name Muscular Dystrophy Research Foundation. Immediately, she received a letter from the executive director of the Muscular Dystrophy Association, Robert Ross, accusing her of deliberately choosing a name that would confuse the public. “He was wrong,” she says. “I just wasn’t very creative.” She changed the name to Parent Project Muscular Dystrophy.

The twenty or so parents who made up the group held bake sales and dinner dances and canvassed family, friends, and neighbors for donations. “For one holiday season, we were baking and sending out holiday cards to our donors,” Furlong says. “I devised this holiday card full of sparkles and set up an assembly line in my kitchen, someone adding the glue to the card, the next person adding the sparkles.” Her motivation in starting the parent group was not sadness but anger, she told me: “at God, at the world, at being in Middletown, Ohio, not having the academic resources at my fingertips, having doctors who say I’m crazy, angry at a husband who doesn’t seem to be in synch with me, angry at myself for a genetic disease.”

In 1986, Louis Kunkel, a research scientist at Boston University, isolated the gene implicated in Duchenne. This gene—by far the largest in the genome, with 2.3 million base pairs—is responsible for making a protein that
Kunkel and his collaborators named dystrophin. The protein is found in every muscle of the body; it forms a shock absorber around the membrane that surrounds muscle cells and holds them together. Owing to errors in their genetic code, Duchenne boys do not produce dystrophin. Over time, the cell membranes rip, and calcium floods into the cells. The immune system, mistaking the activity for an invading pathogen, attacks the cells and kills them. Muscles fail progressively throughout the body: first the large skeletal muscles of the legs and arms and trunk, then the muscles of the diaphragm, digestive tract, and heart.

In the early nineties, Furlong began visiting gene-therapy researchers, including Eric Hoffman, a geneticist who had worked in Kunkel’s lab and then established an independent laboratory at the University of Pittsburgh. Furlong asked Hoffman to address the members of Parent Project, whom she was bringing together for an information-sharing meeting in Ontario, in the spring of 1994. Furlong says that many researchers were leery of associating with Parent Project out of fear, “real or imagined,” that the Muscular Dystrophy Association would cancel their funding. But, she says, “if Eric Hoffman agreed to come, we knew the rest would come along.”

Hoffman, however, declined the invitation. “He said, ‘No one’s going to come,’ ” Furlong recalls. “What would incentivize us?” On a table in Hoffman’s office was a copy of Time, with a cover story on the geneticist French Anderson, who had performed the world’s first successful gene therapy, on children with ADA deficiency, an immune disorder. In desperation, Furlong grabbed the magazine and said, “He’s coming.” Hoffman was clearly dubious. “He just looked at me, like, ‘You’re the dumbest shit I’ve ever come across.’ ”

Furlong immediately flew to Pasadena, where Anderson had an office at U.S.C., and installed herself in his waiting room until he emerged. “I told him the whole story,” she says. “I’m in tears: ‘You have to do this. I made up a big lie, oh, my gosh—I’m crazy, I’m a liar, but you have to come!’ He just said, ‘O.K.’ ” Furlong shrugged. “I think he was afraid of me.”

At the meeting in Ontario, Anderson (who, in 2006, was convicted of sexual abuse of a minor and is currently serving a fourteen-year prison sentence) announced to a crowd of parents, patients, and scientists that he would cure Duchenne in eighteen months—a grandiose claim that almost no one in the room believed. But Furlong had managed to assemble several of the world’s leading experts on the disease, including Hoffman. “The meeting galvanized everyone,” Furlong says. “I recall talking to the researchers after the meeting. They were pretty amazed that parents were not hysterical, did not expect magic, but wanted to do whatever they could to help. They asked questions about the disease and about us, exploring ideas.”

The parents began raising money to establish a research center. They agreed that the University of Pittsburgh was the logical place; Hoffman was there, and it had a number of “bright minds in muscle and gene therapy,” Furlong says. The parents pledged to raise three hundred thousand dollars. “We had about one hundred and fifty thousand in our accounts,” she says. In the next twelve months, the group raised the rest—through more bake sales, “dinner dances, letters to friends and relatives, and general begging”—and the Duchenne Muscular Dystrophy Research Center, the first of its kind, opened in 1995. Furlong says, “We found a place to land—a place to focus efforts, a place where we no longer felt like ‘just parents.’ ”

Lee Sweeney, the scientific adviser to Parent Project Muscular Dystrophy, has been studying the molecular basis of muscle movement since 1984, and is now the chairman of the Depart-
ment of Physiology at the University of Pennsylvania School of Medicine. He was engaged in gene experiments in Duchenne using mice when, in the mid-nineties, Furlong sought him out and offered funding. “Parents, the minute they find the disease, do one of several things,” Sweeney told me. “Either they withdraw and just feel like the world has attacked them or they decide that they’re going to do something about it, and attack back. Often they do what Pat did, early on, which is to try to form some charity—usually in the name of the child—and they go out and raise money from their friends and other people.” But developing a drug from initial lab experiments to F.D.A. approval costs hundreds of millions of dollars—far more than grass-roots charities can raise. “When I first met Pat, I thought, It’s another one of these people, and I feel badly for her,” he said. He turned down her offer. “The type of money she was talking about was relatively small,” he said, and the gene approaches he was working on were far from yielding any benefit to humans. But Sweeney agreed to speak at an annual meeting of Parent Project Muscular Dystrophy.

In October, 1995, the Furlongs’ older son, Christopher, then seventeen, caught a cold that developed rapidly into pneumonia—a common occurrence in Duchenne boys, whose weak diaphragms make it impossible for them to clear their lungs by coughing. (Furlong has since worked with the pediatric pulmonologist Jonathan Finder to include the mechanical insufflator-exsufflator—a device that aids in coughing—in the standard care for Duchenne.) Christopher was admitted to the hospital, intubated, and placed on a respirator. His condition worsened, and, after two weeks, he died.

Seven months later, Patrick, too, got a cold, and his health declined. “After Chris died, I think Patrick gave up,” Furlong told me. “He watched his brother lose strength and die. He knew. Patrick and I could barely look eye to eye without tears. It was as if we knew a secret and if one or the other said it out loud it would come true.” Furlong took Patrick to the hospital and stayed with him all night. In the morning, he said that she should go home and shower, and asked that she get his father to spell her. He joked that “having a doctor around was much better than a nurse.” Furlong summoned Tom, then drove home. She was at the door of their house when Tom called her cell phone to tell her that Patrick’s lungs and heart had failed.

Few parents, Sweeney says, remain committed to fighting Duchenne after their child’s death: “I thought that, like most of the parents, Pat would never want to hear the name of the disease again—she would disappear, her organization would disappear, and that would be it. But, to my surprise, if anything she came back to me even more aggressive.” Furlong says that she never contemplated quitting. “I don’t understand how you can close the door and say, This part of my life didn’t exist,” she told me. She was also inspired by something that Christopher said to her shortly after he and his brother received their diagnosis of Duchenne.

“We were in the kitchen and I was getting ready for dinner,” Furlong said. “I must have been crying, Chris said to me, Why are you upset? and I said, I just want you two cured. And he said, Do you think it’s fair if it’s just Pat and me? Just in a very weird kind of kid-like way. Wouldn’t you want everyone cured? Aren’t there more kids like this?”

One of Sweeney’s first acts as scientific adviser to Parent Project was to speak frankly to its members about the ineffectiveness of their approach. “At a meeting, I told them, just flat out, You know, this raising money from your friends and from bake sales is never going to get you research dollars that are going to have any impact. Maybe it makes you feel better, but it’s not going to do anything.” My advice to them was to use the power of parents lobbying for children—which is a pretty powerful message—with their congressmen.

Furlong had been trying for years to make headway in Washington. “Right after the boys were diagnosed, I was at the N.I.H. asking, What are you doing?” Furlong told me. “The answer was nothing.” She was introduced to Steve Groft, at the Office of Rare Diseases Research, and began visiting
his office, which she describes as "Mother Teresa’s waiting room of rare diseases." Many concerned parents came to ask for help, she says, "but I was a pretty persistent one, going back, and calling Steve and saying, What are we doing here? I am, by nature, a pest."

Furlong’s unusual knowledge of the disease made her persuasive, Groff said. "With her training in nursing, she had a very good understanding of diseases and the needs of patients and families, and, as a mother of patients, she knew very well what her needs were." But his office by itself could be of limited help. "Our office was not very large," Groff explained. "I think we had two people." Following Groff’s advice, Furlong met with the directors of several divisions of the N.I.H. that were potentially relevant to Duchenne, and with Dr. Harold Varmus, the director of the agency. Furlong recalled that in one meeting someone said, "We have heard about you, Mrs. Furlong." She considered that a victory. In 2000, after six years of appeals, she received a commitment from the N.I.H. to fund a "workshop" in Duchenne—a two-day conclave of researchers whose areas of focus were related to the disease. But when she told Sweeney about the workshop he was unimpressed. "He said, They’re going to give you a workshop because you’re in the queue," Furlong told me. "I’m here’s your workshop, now go away. It’s not enough." I realized he was right.

Furlong had recently met a Washington lobbyist named Joel Wood, who had a son with Duchenne. Wood put her in touch with B&D Consulting, a lobbying firm with expertise in pediatrinc legislation. Furlong and others from Parent Project worked with the lobbyists on a proposed bill that would compel the N.I.H. to fund a number of research centers for muscular dystrophy, with two devoted to Duchenne. Furlong then spent months visiting the office of Bettilou Taylor, the staff director of the Senate subcommittee in charge of health appropriations, to press for a hearing. Furlong describes Taylor as a formidable figure: "She was in her St. John’s perfect outfits and the hair perfect and the makeup perfect and the Nancy Pelosi perfect face with the perfect smile and the sort of ‘Tell me your story, Yes, I understand, I care a lot—next!’" Taylor was unable to schedule a hearing. Then, one day in late 2000, Furlong arrived with another Duchenne mother. Before they met with Taylor, Furlong told her not to sob; they had to be professional. "We went into this little conference room," Furlong recalls, "and she put her arms around Bettilou—put her tears on Bettilou’s St. John jacket, or Armani or whatever—and I thought, Dear God, this is the end. And she just went, ‘You have to save my son, he’s going to die!’ And Bettilou said, ‘I’ll do whatever you want,’ and started crying.”

In February, 2001, Congress held the hearing, with Senators Arlen Specter and Paul Wellstone leading the panel. The gallery was filled with muscular-dystrophy patients and their families. An N.I.H. director testified about what the agency had been doing for Duchenne—which was, the senators learned, not very much. "I’m sure it was very unpleasant," Sweeney, who also spoke that day, says. "It looked very unpleasant from where I was sitting.” Furlong, too, testified before the panel. Speaking in a strong voice, she told the senators that Duchenne "gets only one-one-thousandth of the N.I.H. budget" and called for an investment of a hundred million dollars over the next five years. She also told a brief but potent anecdote: "One day long ago, my son Patrick was trying to convince me of a crazy argument he had. He said to me, ‘Mom, pretend I’m in a midlife crisis.’ In fact, he was. He was eight." The bill passed the Senate and the House that spring, and in December, 2001, President Bush signed the Muscular Dystrophy CARE Act into law.

"That changed the landscape entirely," Sweeney told me. With millions of dollars available for research, scientists began to concentrate their attention on Duchenne. In 2000, there were fewer than ten trials and studies on Duchenne in process; there are now more than fifty. Parent Project has a membership of three thousand families in the United States, with affiliates throughout Europe, and an annual budget of five million dollars. Because the organization still has relatively small amounts of money to spend—usually two hundred thousand to three hundred thousand dollars for any given treatment—Sweeney and Furlong often invest in small laboratories or drug companies that need startup capital to bring a drug to a point where it can attract larger investment.

In 2007, Parent Project began working with a small company in New Jersey, PTC Therapeutics, which was developing a drug called ataluren, intended for boys whose Duchenne is the result of a "nonsense mutation" in the gene—a period in the genetic code’s sentence, which renders it unreadable by the body’s protein-making machinery. Furlong first learned of the company, from Sweeney, in 2000, and offered to help fund development of the drug. But the mutation addressed by ataluren accounted for only about fifteen per cent of all Duchenne sufferers. Stuart Peltz, the C.E.O. of PTC Therapeutics, told me, "After a couple of years working together, Pat said to me, ‘I love what you’re doing for the fifteen per cent of the patients—you’ve got to do something for the rest.’" Furlong said that she would raise a million dollars from Parent Project’s membership to support the company’s efforts to find other targets on the gene. Four promising new targets turned up, and the N.I.H. awarded PTC Therapeutics and Parent Project, along with the University of Pennsylvania, $15.4 million for further research. Peltz says that the partnership between a drug company, a patient-advocacy group, and government "set up a new paradigm in how to fund drugs for these rare diseases."

In April, 2008, ataluren went to blind clinical trial, with a hundred and seventy-four boys at clinics in North America, Europe, Australia, and Israel. Two groups of boys were given the drug, in a high and a low dose, and a third group was given a placebo. Over forty-eight weeks, their progress was
monitored by six-minute walk tests, a standard measure of muscle function in Duchenne boys. To the disappointment of everyone involved, boys on the high dose of ataluren declined at the same rate as boys not on the drug. The clinical trial was suspended. But researchers noted that boys on the lower dose did not decline in function. This tallied with preclinical experiments on cell cultures, which showed that if the dose is too high the drug’s effect shuts off. “We initially discounted this as some artifact of cell culture and not real,” Sweeney says. Furlong has advocated for patients to be allowed to take the drug as part of an F.D.A.-approved “access program,” which permits terminal patients to take an experimental drug when no other treatment is available.

Sweeney and Furlong are optimistic that it will benefit the boys. Furlong, who has seen more than a thousand Duchenne patients, and who, according to Sweeney, “understands the disease a lot better than a lot of the clinicians and scientists who work on it,” is convinced that she noticed an effect in boys on ataluren. “I am well aware there could be a placebo effect,” she says, “but I saw changes, a posture that changed, a gait that improved, energy.” Sweeney, though, believes that a cure is decades away and will likely involve stem-cell therapy. “It’s not just fixing one muscle,” he told me. “It’s fixing every muscle in the body. That’s the problem. Getting the cells to the right place and then getting them to do the right thing—it’s a daunting engineering problem as much as anything else.” For now, he says, researchers are focused on developing multiple therapies that address various pathways of the disease. “It’s the same sort of idea as with AIDS, where you have a combination of drugs, where none of them cure you but they can hold the disease at bay quite successfully with many patients. That’s the goal here.”

One morning in early November, Furlong had a meeting at Cincinnati Children’s Hospital, where her sons originally received their diagnosis. After that, Furlong had not returned to Cincinnati Children’s for almost a decade, but in 1999 the hospital hired a new pediatric neurologist, Dr. Brenda Wong, and Furlong began to meet with her regularly. She urged Wong to build a department that would focus on multidisciplinary care and treatment for Duchenne boys—epidemiologists to care for hormonal problems that arise with steroid treatment, lung specialists, immunologists, cardiologists. Thanks in part to Furlong’s efforts, which included fundraising for the hospital, Cincinnati Children’s is now the leading clinical treatment center for Duchenne in the country. “I’m happy that this happened here,” Furlong told me, when I met with her at the hospital. “Because it’s payback.”

Furlong was sitting in the airy cafeteria with a pediatric cardiologist, Dr. Linda Cripe, an open-faced blond woman in her forties. They became acquainted almost ten years ago, when Furlong called Cripe to persuade her to attend Parent Project’s annual meeting, in Pittsburgh. The day before the meeting, Cripe’s flight was cancelled because of bad weather, but, having promised Furlong she would be there, she drove from Cincinnati to Pittsburgh overnight. “I’m cursing her the entire way,” Cripe told me. “I’m so angry, saying, ‘Who is this woman? She’s making me go to this thing . . .’”

Doctors who attend Furlong’s conferences often report a kind of conversion experience. Jonathan Finder, the pulmonologist, addressed the Parent Project’s annual meeting in 1999. He told me, “It was one of those things that are hard to describe, the emotional impact of being surrounded by parents. As a physician, you meet with parents one at a time; you don’t go into rooms filled with them.” Cripe, at her first conference, spoke to a crowd of three hundred and fifty parents. Afterward, she called the hospital’s other senior cardiologist and persuaded him to focus more on Duchenne patients. “Pat forced me to go into a situation that I didn’t want to be in,” Cripe says. “She brought the problem to me. I think if you’re paying attention in medicine you’d have to be deaf, dumb, and blind not to see that there was a message there.”

Three years ago, Cripe was instrumental in bringing about one of the first pediatric heart transplants given to a Duchenne child. During my visit, the recipient of the transplant, Riley Herrera, from Helena, Montana, was at Cincinnati Children’s for a follow-up, and Furlong met him and his father, Ron, for lunch. Ron, a federal law enforcement agent, is a trim, muscular
man with a military bearing. Riley has dark hair, braces on his teeth, and a shy smile. He has been on steroids since the age of seven, and at eighteen is smooth-faced and under five feet tall; he might have been mistaken for a twelve-year-old. He was still walking, albeit with the characteristic Duchenne gait; we went to lunch at a nearby Mexican restaurant, and his father had to lift him up the two stairs that led inside.

At lunch, Ron asked about the latest developments in treatment. "The myostatin—is that a good one?" he said. I was referring to a drug, being developed by a company called Acceleron Pharma, that promotes muscle growth by inhibiting the uptake of myostatin, a protein that the body produces to prevent muscles from growing too large.

"Well, we'll see what Acceleron does, right?" Furlong said, carefully. With parents, she dispenses what she calls "therapeutic doses of hope" but does not promise too much. The drug is still in the early stages of testing and far from approval by the F.D.A.

"I saw a video of that dog that had the exon-skipping," Ron said. A recent YouTube clip showed a puppy that had been treated with a drug for a Duchenne variety caused by gaps in the code of the dystrophin gene. The drug had shown some success in dogs, but it would be years before it could be used in humans.

"Oh, yeah," Furlong said, in a neutral voice.

Talk turned to Riley's heart transplant. In 2006, during a family trip to New York, Riley had turned pale and become too tired to walk. "I'd get stabbing pains in my stomach," Riley recalled. "It was hard to breathe." When the family got home, Ron took him to a cardiologist in Billings, who said that Riley's heart was failing and that only a transplant could save him. Ron, who had been a member of Parent Project since 1999, turned to Furlong. She approached Cincinnati Children's, but there was controversy about giving a transplant to a "terminal" child, since pediatric hearts were in such short supply. Cripe argued that Riley should be on the transplant list. "The definition of Duchenne includes loss of ambulation between eight and ten years," Furlong explained. "Riley was fourteen and walking, so by clinical definition he was not a typical Duchenne." The hospital agreed to put Riley on the list, and Furlong found a donor to pay for him and his family to live in Cincinnati while they waited for a pediatric heart to become available.

Riley arrived in Cincinnati in January, 2007. Three months later, no heart had arrived. Furlong, who visited Riley in the hospital one Friday at the end of March, knew that he was running out of time. His heart was so enlarged that she could see it beating beneath his rib cage, through his shirt. This time, there was nothing that she could do.

"You said, 'You're going to need a heart in the next day or two,'" Ron said to Furlong. "And it happened—I think the next day?"

"The next day," Furlong said. "It was Friday. The heart came Saturday."

Riley was looking closely at Furlong during this exchange, apparently hearing for the first time how desperate his situation had been. Had it not been for Cripe—and for Furlong's long campaign to reform Cincinnati Children's Hospital—he would almost certainly have died. He said, "So—you're like an angel."


After lunch, Furlong was due to drive home to Middletown. Despite years of difficulty, she and Tom have repaired their relationship. "He knows why I do what I do and is happy for progress," Furlong says, "but it is still an open wound. I think he knew that this family needed both of us, even if we hated each other for a time." Before Furlong left, I asked her about Parent Project's long-term goals. "It's about looking at the landscape and seeing where you can play in this chess match, see how you can maximize the impact on research, see how you can apply that directly and quickly to the kids," she said. "From the day my sons were diagnosed, I wanted to buy time. So we look at a research opportunity and say, 'What is going to buy us time? What do you think we could tweak, invest in, move, that would buy us more time?'"