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Months to Live

Fighting for a Last Chance at Life

By [AMY HARMON](#)

VIRGINIA BEACH — As [Lou Gehrig's disease](#) sapped Joshua Thompson of his ability to move and speak last fall, he consistently summoned one question from within the prison of his own body. “Iplex,” he asked, in a whisper that pierced his mother’s heart. “When?”

Iplex had never been tested in people with amyotrophic lateral sclerosis, the formal name for the fatal disease that had struck Joshua, 34, in late 2006. Developed for a different condition and banished from the market by a patent dispute, it was not for sale to the public anywhere in the world.

But Kathy Thompson had vowed to get it for her son. On the Internet, she had found enthusiastic reviews from [A.L.S.](#) patients who had finagled a prescription for Iplex when it was available, along with speculation by leading researchers as to why it might slow the progressive [paralysis](#) that marks the disease. And for months, as she begged and bullied biotechnology companies, members of Congress, Italian doctors and federal drug regulators, she answered Joshua the same way:

“Soon,” she said. “Soon.”

At a time when terminally ill patients have more access to medical research than ever before, and perhaps a deeper conviction in its ability to cure them, [many are campaigning](#) for the [chance to be treated](#) with drugs whose safety and effectiveness is not yet known.

But even as advances in areas like [stem cells](#) and [genetics](#) generate greater hope for experimental therapies, there is little consensus on how and when to provide them to dying patients whose lives could be prolonged, or shortened, by trying them.

Insurance companies typically do not pay for drugs that are part of a not-quite-finished scientific process. But even affluent families like the Thompsons find themselves pleading simply for the right to buy a drug, with institutions and individuals that often seem to them to have no logic — and sometimes no heart.

Doctors worry about instilling false hope and doing unnecessary harm. Companies fear damaging a drug’s chance of winning approval from the [Food and Drug Administration](#) if a patient suffers a bad reaction. The F.D.A. itself does not want patients to bypass clinical trials, which require that some participants receive a placebo to determine reliably whether a drug works.

[Some patient advocates](#) are lobbying for [laws](#) and [policies](#) that would sanction what has become known as the “[compassionate use](#)” of experimental drugs by seriously ill patients who have run out of other options. But for now, each appeal to the guardians of untested drugs is an improvisation, in which success relies on connections, determination, mercy and luck, and the hope of prevailing can sometimes eclipse the hope held out for the drug itself.

The Search for a Treatment

Kathy discovered Iplex deep in the pages of her first Google search for “A.L.S. and treatment” late one night in spring 2007, shortly after Joshua’s diagnosis.

In the daylight, she still had trouble believing that her athletic, magnetic son had the devastating disease with an unknown cause, named for the 1930s New York Yankees star whose career was cut short by it. When Joshua’s racquetball racket flew out of his hand because he could not grip it, his mother’s diagnosis was [tennis elbow](#). When the first neurologist mentioned A.L.S., she scheduled more tests, rooting for Lyme’s disease, [multiple sclerosis](#) or even [cancer](#).

But an A.L.S. specialist at [Johns Hopkins University](#), Dr. Jeffrey D. Rothstein, had confirmed it. Like the 5,600 other people given the diagnosis each year in the United States, Dr. Rothstein said, Joshua would almost certainly die of the disease in two to five years.

The doctor prescribed the only drug approved for A.L.S., Rilutek, which typically prolongs life by a few months. Joshua, he said, would be eligible to participate in a clinical trial for another drug, Arimoclomol, that would start as soon as the F.D.A. gave the go-ahead. There was nothing else.

Except, maybe, Iplex.

Kathy clicked her way through [online news releases](#), [blogs](#) and [scientific journal articles](#). Iplex, she learned, is believed to protect the motor neurons whose death leads to paralysis in A.L.S. Some patients had persuaded their doctors to prescribe the drug when the F.D.A. approved it in late 2006 for children with growth deficiencies.

“I started on Tuesday,” Debbie Gattoni, an A.L.S. patient in New Jersey, had written on a [Web discussion forum](#), “and on Sunday, I noticed that my right index finger, which was bent, was straightening and moving on its own.”

But almost immediately, the drug’s maker, Inmed, lost a patent infringement lawsuit to a biotechnology firm that was already selling a drug for [short stature](#) that had similar properties. Iplex, however, was thought to be more potent for treating A.L.S.

Inmed agreed to pull its drug off the market. Only the [Italian Health Ministry](#), which had begun to distribute the drug to A.L.S. patients under a compassionate use program, could continue to buy it.

Kathy dashed off a letter to the F.D.A.

“Is there any way we can get Iplex,” she wrote, “before it is too late for my son and others like him?”

But the agency could not weigh in until Insmmed agreed to make the drug available. And Insmmed’s hands were tied by the settlement agreement.

Mounting Physical Needs

In late July, Joshua fell in the street near Times Square on a trip to New York with his wife, Joy, and could not get up. Joy could not lift him, and passers-by did not stop. Finally, a homeless person watching from the corner came to help.

The event was traumatic for Joshua, who was beginning to experience another symptom of the disease, too, a lack of control over his emotions. At the weekly poker game with his friends, he could no longer bluff.

As Joy helped Joshua with his mounting physical needs and cared for their son, Wyatt, who had been born two weeks after Joshua’s diagnosis, the couple pressed Kathy to explore Joshua’s medical options.

They decided he should try what seemed like the next best thing to Iplex — the drug that had triumphed over it in the patent dispute. But the first neurologist they visited refused to give him a prescription.

“This could cause [hypoglycemia](#),” he told Joshua, warning that low blood sugar could result in [seizures](#) or brain damage.

“I’ll take my chances of hypoglycemia over laying in the gutter,” Joshua replied fiercely, but the doctor did not relent.

Kathy found another doctor to prescribe the drug, called Increlex. But she cried later when she read a [blog entry](#) by an A.L.S. patient who said he had experienced a “seismic” improvement on Iplex before it was withdrawn. The Increlex shots he was taking now felt like “trying to get drunk on [cough](#) syrup when there’s a case of bourbon locked in the closet,” he wrote.

At brunch with her long-term boyfriend, Richard Stravitz, soon after, Kathy told him she did not want to play golf that afternoon, as was their Sunday ritual. As always these days, she could think only about trying to help her son.

“I understand,” she said, “if you need to move on.”

Mr. Stravitz, a sculptor and retired chairman of the meat processing giant Boar’s Head Provisions, shook his head.

“I want to help,” he said.

Involuntary twitches known as fasciculations signaled which of Joshua's muscles would be the next to go. His mother watched the disease spread from his right arm to his left arm to his left leg. A natural storyteller who had played toastmaster at friends' weddings and charmed business associates with stories of his misadventures in surfing and snowboarding, Joshua began to slur his words in what is known as the "A.L.S. accent."

Kathy, who had majored in biology before leaving college when she was pregnant with her son, ruled out traveling to [Belize or China](#) for stem cell infusions with no proven value. But she did pay \$25,000 to send Joshua to a holistic health program in Arizona for a month. At the clinic, he had [physical therapy](#) and swallowed vitamin cocktails. He spent hours each day hooked to an intravenous tube to remove heavy metals from his body. But any improvement quickly dissipated on his return.

A few weeks later, the [cancellation of the clinical trial](#) that Joshua had been waiting to participate in came as a sharp blow. The F.D.A., the Thompsons were informed, wanted more animal research first.

"We have to get Iplex," Kathy told Joshua's father, Bruce Thompson. "I'm going to find a way."

Divorced 20 years earlier, the Thompsons still sometimes shared holidays and vacations with Joshua and their younger son, Christopher, but they disagreed on how to approach A.L.S. Bruce had concluded there was little to be done medically. He proposed a trip to Europe, where Joshua had never been.

A hotel and restaurant developer who had been training his older son to someday take over his business empire, Bruce remodeled his guest house, where Joshua and his family had moved, to make it wheelchair accessible. And in December, tracing Joshua's [irritability](#) and depression to the cycle of raised and dashed hopes, he admonished Kathy not to mention to their son what seemed like an impossible long shot.

"Let him live his life," Bruce urged her in a heated conversation. "Don't give him false hope."

"This isn't false hope," Kathy retorted. "It's real. And it's all we have."

Joshua did not go to Europe. But in February, he and Joy told their inner circle and that Joy was pregnant with their second child. Wyatt, the couple had decided, should have a sibling.

On hearing the unexpected news, one family friend gave Joshua a fist-bump.

"I didn't know you could do that," he said.

Joshua laughed.

"It seems to be the only thing that's still working," he replied.

'Our Rights to Live'

Adopting the online moniker “FightingMom,” Kathy haunted Web forums devoted to the disease. At night, after working full days at her own business, which manufactures filling for quilts, she exchanged messages with several dozen patients and caregivers who came to call themselves “Team Iplex.”

“Fighting Mom,” wrote Andrea Reimers, a nurse who was pursuing Iplex for her husband, Jim. “It seems to me that there haven’t been enough cries from the A.L.S. community to demand our rights to live.”

Mrs. Reimers thought a demonstration by dying people in wheelchairs might shake up the companies holding Iplex under wraps.

Her militancy emboldened Kathy, whose letters and calls to public officials took on a more insistent tone. But everyone told her it was a corporate matter.

She also turned her attention to Italy, where the government was still spending several million dollars a year to buy Iplex for A.L.S. patients. A doctor there wrote to Kathy that she had seen “very good results in almost 50 percent” of the A.L.S. patients she had treated with Iplex over two years. Joshua and Joy were ready to move there, but the answer came back: only Italian citizens could receive Iplex through the program.

“Josh’s sadness is unbearable,” his mother wrote one night in her journal, nearly a year after her son’s diagnosis.

Unexpected encouragement came in a Mother’s Day note from her ex-husband. “You have given me some peace of mind that all potential options for Josh are being researched and acted upon,” Bruce wrote. “Thank you.”

Kathy’s boyfriend accompanied her to Insmed’s headquarters in Richmond, Va., offering to raise several million dollars to underwrite a compassionate use program for Iplex in the United States with A.L.S. patients. But the couple came away with a new understanding: F.D.A. regulations, they were told, prohibit any company from profiting on compassionate use. Even if Insmed could wriggle free of restrictions in the patent agreement, there was little financial incentive for it to invest in making the drug solely for compassionate use by A.L.S. patients.

Kathy was not surprised when weeks passed without hearing back.

Seizing an Opening

By July, Joshua could no longer lift his arms. Feeding him one evening, Kathy stifled a cry when she saw his tongue twitching as if it had large worms crawling inside it. Worse for her was the knowledge that he had felt the fasciculations, and known what they signaled: the end of swallowing, saliva control and speech.

“I can’t believe I may never hear him speak again,” she wrote in her journal.

The best hope for regenerating the long nerves that control muscle movement, Kathy knew, were [stem cell therapies](#) still in very early stages of development. But if Iplex worked as hoped, it would slow the death of those nerves, perhaps sustaining Joshua's life long enough for a cure to come along.

That fall, a major study at the [Mayo Clinic](#) concluded that a hormone that is the active agent in Increlex and Iplex [had no effect](#) on people with A.L.S. But a leading researcher suggested that a drug like Iplex, which combined the hormone with a protein that could deliver it to cells more effectively, could have better results.

Kathy saw an opening. Genentech, the company that held the patent for Increlex, would now most likely have no market for its own drug among A.L.S. patients. Perhaps it would release Iplex from the restrictions of the patent settlement.

Aggie Wilson, the assistant who answered Kathy's call to Genentech's chief executive, listened to her story.

"His son keeps sitting on his lap saying 'Hello, Dada,' waiting for Josh to say hello," Kathy said of Wyatt.

Ms. Wilson told Kathy that if she wrote a letter, she would personally deliver it to her boss.

Kathy did not wait for his response. The members of Team Iplex [set a date for the protest: Veterans Day](#), at the Capitol in Washington. They let the drug companies know that dozens of news media outlets had been alerted.

Joy gave birth to a boy, Jordan, a few weeks later. And at the urging of his mother and his wife, Joshua assented to using a machine that enabled him to communicate with a speech synthesizer. A camera picks up small movements of his head as he "types" letters on a screen, tracking a reflective patch on his forehead.

Kathy and Joy watched him laboriously peck out his first words, anticipating an expression of gratitude or affection. Instead, the machine pronounced a playfully vulgar directive. Gleeful, Joshua hit the "repeat" button over and over.

The Saturday evening before the protest, Kathy [found a letter in her e-mail](#). The competing companies had agreed to make Iplex available to A.L.S. patients worldwide on a compassionate use basis, pending the approval of the regulatory agencies.

On a crisp fall day, on the lawn outside the Capitol, Team Iplex handed out information about the disease, and celebrated. All they needed now was for their doctors to file a formal request for the drug with the F.D.A.

Safety Concerns

Joshua's doctor at Johns Hopkins, Jeffrey Rothstein, believed there was a rational logic for why Iplex might work. But the review procedure at Hopkins, he told Kathy, could delay getting the request to the drug agency. Instead, Kathy took the stack of paperwork to her son's local family practitioner, Dr. David L. Werwath, only to burst out crying in his office when he demurred.

He had never treated anyone with A.L.S., much less with an experimental drug.

But that evening, he called Kathy on her cellphone to tell her he had had a change of heart.

"Look, I'm a father," he said "If it were my son or daughter, I'd be doing the same thing."

The F.D.A. had a month to respond from the date it received Joshua's application on Dec. 16. When Joshua asked about Iplex now, Kathy told him, "It should be any day."

On Jan. 16, when Dr. Werwath called to tell her the application had been rejected, she stood up in disbelief.

"How could that be?" she asked, dazed.

Kathy's friend Mrs. Reimers had received a call with the same news.

"He said they had safety concerns," Mrs. Reimers said. "This for a drug that was approved for children!"

"Safety," Kathy repeated. "And what, exactly, is safe about A.L.S.?"

Appealing an F.D.A. Denial

Before the F.D.A.'s decision, Kathy had spared little thought for any broader meaning of her quest for Joshua. But when she met with Richard A. Samp, a lawyer with the [Washington Legal Foundation](#) a week later, her outrage went beyond her son, and beyond Iplex.

"The F.D.A. is supposed to protect American citizens," Kathy fumed over an iced tea in Williamsburg, Va. "How does denying dying patients access to this drug serve the common good?"

Mr. Samp had handled a lawsuit by a patient advocacy group, the [Abigail Alliance](#), that had sought to establish a constitutional right for terminally ill patients to use experimental drugs. In the case, which the group [had lost on appeal in 2007](#), the F.D.A. claimed that it granted "nearly all" requests for compassionate use.

They would first make an administrative appeal, Mr. Samp told Kathy, asserting that the F.D.A. had violated its own guidelines. If that failed, they could pursue litigation that might allow them to raise the constitutional question again in a federal court in Virginia.

"But," he said carefully, "that might continue for a number of years."

Because a lawsuit cannot continue if the plaintiff dies, Mr. Samp explained that it might require forming an organization to carry it forward.

Kathy stirred her iced tea.

“I’d like to do that,” she said finally. “This case could be important to lots of patients, even if Josh is no longer involved.”

In its follow-up letter, the F.D.A. had indicated that its chief concern was that “adequately controlled trials would become virtually impossible” if it granted requests like Joshua’s. In the appeal, Mr. Samp argued that Joshua and other A.L.S. patients, whose life expectancy was measured in months, would never have an opportunity to participate in such a trial.

Kathy had avoided telling her son that the drug agency had turned them down. His swallowing had deteriorated to the point that he choked after just two sips of an Orange Crush soda he asked his mother to bring him one night. When he finally agreed to have a feeding tube inserted in mid-February, his family viewed it as a statement that he wanted to live. But the tube also represented a new frontier in the heartbreak of A.L.S., which took away small pleasures every day, and sometimes big ones.

“How cruel can you be?” Bruce barked at his younger son, Christopher, when he unwrapped a hoagie in front of Joshua.

Mr. Samp’s foundation, a conservative public interest law firm, had agreed to work on the appeal pro bono for members of Team Iplex. Kathy tried to interest Andrea Reimers in joining her appeal. But after learning of the drug agency’s decision, Mrs. Reimers said, her husband went into respiratory distress and now could barely breathe.

He died on [Valentine’s Day](#).

Winning a Reversal

Kathy was pouring milk for her cereal on the morning of March 10 when Dr. Werwath’s number flashed on her phone. The F.D.A. had just reversed itself, he said.

Before she could take a breath, Senator Mark Warner’s office called. E-mail bleeped in as the news seeped out.

In the weeks after the appeal, Kathy learned, the F.D.A. had reached out to Insmmed. The agency had persuaded the company to run a clinical trial for Iplex with several dozen A.L.S. patients, and permitted it to recoup the hefty costs directly from participants. In the trial, some of the participants would get a placebo. That way, the F.D.A. [wrote on its Web site](#), the next wave of A.L.S. patients would learn whether the drug was in fact beneficial or harmful.

But for now, the agency had ruled, Joshua and 12 other patients would be given Iplex outside of the trial, on a compassionate use basis, if they agreed to read [all the data about the risks](#).

Who would pay for the drug, which Insmad said could cost \$100,000 a year, was unclear.

Kathy called her daughter-in-law Joy and asked her to turn the speakerphone on. She reported the news, and smiled to hear Joy tell her that Joshua had raised his eyebrows, his sign of approval. Then she sat down at her kitchen table and gazed out at the ocean.

“Oh my gosh,” she thought for the first time. “What if it doesn’t help him?”

Dr. Werwath arrived with Joshua’s first vial of Iplex at 9:10 a.m. on Wednesday, March 25.

The doctor slipped on his gloves and showed Kathy and Joy how to extract the medicine with a needle. Then the women followed the doctor into Joshua’s bedroom and watched him give the injection, which one of them would do daily.

Joshua slept most of the day.

But when Kathy visited a few days later, her son was sitting in a reclining chair in the living room, in good spirits. They watched the end of a college basketball game together. Around 11:30 the original “Airplane” movie came on.

Joshua smiled a lot. It made her smile too.

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EPILOGUE: In April, Joshua signaled that he felt he was swallowing better, and to Joy and Kathy he seemed to be regaining tone in his voice. But on Easter Sunday, he was rushed to the hospital with [pneumonia](#) and is still on a ventilator. He continues to take Iplex every day.