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Cases highlight ethical dilemma

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Two girls -- Dakota Bihn of Olmsted Falls and Alexis Markowich of Amherst -- are afflicted with juvenile Tay-Sachs disease, a rare and fatal inherited condition. They spent months at Duke University Medical Center, where Dr. Joanne Kurtzberg performed a risky and punishing treatment she said might help them. Other experts say her theory is flawed. More than 100 days after their procedures, Dakota and Alexis fight to stay alive.

Durham, N.C. -- Dakota Bihn lay remarkably still on the cold, narrow table of the MRI machine, surrounded by white pillows. She looked as if she were lying in a coffin.

It terrified her father, Ken, for the few seconds he let the image into his head. A brief flash. A way to mentally prepare for what might happen to his little girl if this treatment failed. The treatment he subjected her to.

It had been 121 days since her cord-blood transplant on Jan. 27, and Ken was grateful she was still breathing. Like Dakota, Alexis Markowich, a 4-year-old from Amherst who had had a transplant 150 days earlier for juvenile Tay-Sachs, had improved enough to be released from Duke University Medical Center in Durham, N.C., to a nearby apartment.

Alexis had been back in the hospital with complications. High-dose chemotherapy given before the transplant to wipe out her immune system left her vulnerable to infections. She had had multiple infections in her intestinal tract that caused uncontrollable diarrhea and a rash on her rear end that wouldn't give up. Powerful drugs administered to keep her body from rejecting the transplanted cells affected her kidneys. Fluid had to be drained from around her heart.

A third girl with juvenile Tay-Sachs, 2-year-old Jashaia Small, who had received a cord-blood transplant at Duke 137 days earlier, was back in the hospital having fluid drained from around her heart, too.

For Dakota, there were positives: She was out of the hospital. As scary as it was to leave the safety of having nurses nearby, it was nice to be out of there. The Bihns were renting a two-bedroom apartment down the street from the hospital. It was quieter, and they had their privacy back. But it was not home, where Ken and Bailey, the Bihns' older daughter, were most of the time. Ken had to work, and Bailey, 9, had school.

Dakota was half the girl she was before she arrived at Duke. She was much weaker. She had lost most of her upper-body strength and couldn't walk. She spoke only when cued, one word at a time. Was it the transplant, or the disease marching onward?

Dr. Maria Escolar told the Bihns that the toxicity of the treatment could make Dakota's disease progress more quickly. Escolar, a developmental pediatrician at the University of North Carolina in nearby Chapel Hill, said the procedure might put Dakota into a vegetative state. She might not survive, Ken remembered her saying.

With every complication, Julie, Dakota's mom, thought of Escolar's grim warning.

Julie often wondered what Dakota would be like if she and Ken had decided against the transplant. Would she still be skipping down the halls of Falls-Lenox Primary School? Would she still be giggling and laughing with her friends? Still asking to watch her favorite DVDs: "Cinderella," "Sleeping Beauty" and "Beauty and the Beast" -- fairy tales about girls who live happily ever after?

Julie didn't think Dakota understood most of what went on around her. She looked at her mom as if confused about who she was. Julie never expected Dakota would be this weak.

Dakota was barely eating. What went down came up a minute or two later. Most nights she soiled her pajamas and sheets with diarrhea. Dr. Joanne Kurtzberg, the Duke doctor who did the transplant, wanted to put a feeding tube down her nose. Ken was against it: To him it signaled the beginning of the end. Julie thought a feeding tube was the least of their worries.

Now, as Dakota lay motionless in the cloud of pillows, the MRI knocked and whirred as it took pictures of her brain. Ken sat in the darkened room and watched her chest rise and fall.

The MRI was one of many tests that Kurtzberg ordered about 100 days after a cord-blood transplant. She would compare the results to earlier tests to determine whether the disease had progressed.

Ken didn't need test results to tell him Dakota's condition was spiraling downhill.

The next day was Dakota's 7th birthday. It was going to stink. Ken and Julie didn't feel like celebrating, and Dakota didn't have a clue it was her birthday.

Whether to pursue a transplant for Tay-Sachs, like any other unproven course for a terminally ill child, is a question better answered by medical ethicists than practicing physicians, said Dr. Shawn McCandless, the geneticist at University Hospitals Case Medical Center who made Alexis' diagnosis.

The Bihns and the Markowiches had no good choices: Do nothing, Dakota and Alexis die. Do the transplant, they could die from complications. Or they could survive the procedure and their disease could progress unabated and kill them anyway. What did the parents really risk?

Ethicists say parents are more likely to take a leap of faith when faced with their child's terminal diagnosis. It's hard for parents to say, "I can do nothing."

But ethicists agree that doing nothing often is the right thing to do.

"When a parent in a situation like this says, 'Anything would be better than nothing, because doing nothing is clearly going to result in death in the fairly evident future,' that's not exactly true," said Dr. Norman Fost, director of bioethics at the University of Wisconsin in Madison. "Anything is not better than nothing."

A treatment may result in death anyway, Fost said. It may cause more pain than the child otherwise would experience.

Parents suffer. Their willingness to try something -- anything -- is natural and expected when faced with a child's fatal diagnosis.

"The parents can't be trusted, not because they have a bad intention or want to do anything to harm their child but precisely because they are so desperate to do some good," said Arthur Caplan, a University of Pennsylvania bioethicist. "It's hard for us to understand, but that can be just as distorting as trying to set out to do something bad."

A fine line separates hope and desperation, said Dr. Eric Kodish, chairman of bioethics at the Cleveland Clinic.

"I think hope is a perfectly reasonable and even admirable ethical notion," he said. "But children need to be protected sometimes from their parents' desperation."

Yet, again and again, a symbiotic dance between the desperate and the healer plays out in medicine.

What ethicists say becomes problematic is when a long shot is not reviewed by peers in the medical community, the established process in mainstream medicine.

A peer-reviewed article about transplant survival of children with metabolic storage diseases included a few babies with Tay-Sachs. Another cited research data to demonstrate that older children with different metabolic storage diseases improved after transplants.

It was Kurtzberg's theory that children with juvenile Tay-Sachs also would benefit from treatment. But few subscribe to it.

"I think we all have to remain skeptical of something that's touted as a treatment that no one else has tested and no one else is promoting and no one else is doing," Caplan said.

Using sound scientific methods to demonstrate the validity of an advancement to peers plays an essential role in medicine and in patient protections, he said.

"If you can't convince your peers in medicine, it's different from when you can't convince your peers at, say, the bakery, that the cake you want to make is going to sell," Caplan said.

John Falletta, chairman of Duke's institutional review board, which must approve proposed research on human subjects, said Kurtzberg's transplants on Alexis and Dakota, which she considers therapy and which were not part of a study, complied with hospital policy.

Kurtzberg said in an interview early this year that she turns down hundreds of children with metabolic storage diseases a year, though she said in a later interview that she rarely says no' unless a child was too sick to survive a transplant. Saying "There's nothing I can do" is not what these families need, she said.

"But I know that if you just give up and say, 'This is fatal, go home,' we would never make progress," Kurtzberg said.

The feeding-tube question loomed as a half-dozen nurses gathered in the doorway to sing "Happy Birthday" to Dakota. She was propped up in bed, her legs sticking straight in front of her. She wore her grandmother's birthday present, a denim halter dress and matching hat. Bailey sat beside her.

Dakota smiled weakly at the commotion. The staff gave her a whistle and a picture book from a closet filled with donated toys. Julie had to help her tear off the wrapping paper.

The year before, Dakota's party had been at Chuck E. Cheese. Pizza. Games. Cake. Presents. Ten of her classmates came. She'd had a blast, playing with friends and ripping through presents.

Now, the Bihns were at Duke, waiting hours to see Dr. Kurtzberg at one of the weekly visits they had had since Dakota's release from the hospital. Two weeks earlier, Dakota had stopped eating, became listless and had relentless vomiting and diarrhea. Ken and Julie were anxious about whether Kurtzberg would insist on a feeding tube.

Since the transplant, the Bihns had poured their hope into the weekly visits with Kurtzberg. They hung on every word. Did the latest problems mean the disease was progressing, as Escolar said it would? Or were they more complications to get through, like the nasty rash that began in March and had finally retreated?

Between their visits with the doctor, they gauged Dakota's condition by how Jashaia and Alexis were faring, which was not well.

Alexis was down the hall getting another blood transfusion. Because of constant vomiting, she'd had a feeding tube implanted in her stomach since three weeks after her transplant. Alexis took a nose dive after that surgery.

Alexis hadn't spoken in months. Her belly was bloated, the skin stretched so tight it looked transparent. Her kidneys were impaired, and she had a persistent rash that doctors thought was a symptom of her body rejecting the donated cord-blood cells. She slept most days. When she was awake, she shook wildly and moaned, like a cat in pain.

The parents used Web sites to update family and friends. On the Markowiches' site, Alexis' mother, Nichole, wrote about her daughter's intermittent smiles and giggles between the moans. Alexis tried to move her foot to walk by herself and one day swallowed four times, her mother wrote.

"I was quite excited!!!" Nichole wrote. "It doesn't really take much :)"

Whenever Julie saw Alexis, she was asleep. Or moaning.

After she saw the Smalls' Web site, Julie expected to find Jashaia thriving, and it made her feel jealous. But the next time Julie saw Jashaia, her eyes were closed, her body lifeless.

Julie learned to look at others' Web sites with skepticism. Eventually, she stopped reading them.

Six hours after the nurses sang "Happy Birthday" to Dakota, Dr. K entered the room where the Bihns waited. The doctor noticed Dakota's new outfit. The small talk was brief. Ken started on his list of questions.

"Why do you think she's having trouble swallowing? Could it be a sore throat? Could it be Tay-Sachs?" Ken asked.

"I think it's more likely Tay-Sachs than anything else," Kurtzberg said.

"Really?" Ken said. He was stunned. "It's the disease starting to take away that?"

"Well, I don't . . ." the doctor said, trailing off.

Kurtzberg told the parents it's harder to coordinate swallowing liquids than solid food, so signs of neurological decline would first show up with liquids. Dakota struggled with solids.

Ken was confused. Didn't she just say she thought it was the disease? Maybe he had misheard.

Kurtzberg explained Dakota's options to ensure she was getting adequate nutrition. She recommended a nose tube.

"She's not talking much, either," Julie began. Ken asked over his wife, "How about her other studies?"

Kurtzberg turned the screen of her laptop toward the couple and scrolled through Dakota's test results. No fluid on Dakota's heart. Good news.

Kurtzberg told them Dakota's MRI showed her brain was stable. She pointed out white lines outlining her brain. They could be signs of either a healing brain or a damaged brain, she said. "More likely, it's healing, but we can't prove it on the scan," she said.

"Does that mean that some of the new blood is passing into the brain?" Ken asked.

"Right," the doctor said.

Kurtzberg had told the Bihns that she believed cord-blood cells could cross the protective barrier around the brain, so cells rich with the missing enzyme that characterized Tay-Sachs could start to multiply. This was a point of controversy among other transplant doctors, who said it wasn't necessarily so.

Kurtzberg assured the couple that Dakota was improving and reminded them it would take at least 12 months to know if the transplant had helped.

"Though three years is often the norm in some kids," Kurtzberg said.

Three years? This was the first time Julie remembered hearing her say three years was typical. Julie wasn't sure she could wait that long.

"This is the point you can't sort out which is the disease and which is the transplant," Kurtzberg said.

Ken told Kurtzberg he would feed his daughter protein drinks one dropper at a time, if that's what it took for Dakota to gain weight. He told her he felt a feeding tube was giving up.

Kurtzberg agreed to wait. If Dakota wasn't eating more in two weeks, she would get the tube.

Ken felt better. He thought they had managed to stay one step ahead of the disease.

As soon as Kurtzberg left, Julie turned to Ken and glared. "You sit there and tell Dr. K that we're going to do this, this and this," she said, her right index finger spearing the palm of her left hand.

The feeding tube would eliminate the concentrated liquid nutrition that the medical staff thought was causing Dakota's nonstop vomiting. Julie was exhausted, and Ken was about to board a plane back to Cleveland. "You're leaving, and I'm going to be the one who's up all night, changing

her sheets because she's pooping and throwing up." Tears streamed down Julie's face. She was so frustrated. With Ken. With Kurtzberg. With Duke. With God.

Ken asked Julie to lower her voice. He didn't want the staff to hear them arguing.

Dakota watched her mother's ranting with a fixed, vacant grin. Bailey sat playing her Game Boy. She never looked up.

"I don't know why you're so afraid of the tube," Julie said to Ken. "Is it because it makes it seem like she's so much worse?"

Ken began to pack up for his and Bailey's flight home.

"Dr. K says this will work, and that's good enough for me," he said, zipping his bag shut.

In the five months they had been at Duke, Ken had spent only one week caring for Dakota alone when Julie returned to Olmsted Falls for a visit. Ken didn't have to go to the grocery store, do laundry or clean their apartment.

One time when Julie called home, Ken had forgotten to feed Bailey dinner, and it was after 7 p.m. Most nights he relied on pizza or Kentucky Fried Chicken.

When they decided to go to Duke, neither Ken nor Julie realized the havoc this transplant would wreak on their family life. In the months she'd been here, Julie had missed the Valentine's Day party at Bailey's school, Irish dance recitals, dozens of soccer games and pizza nights, PTA meetings, a spring break trip and scores of good-night kisses.

Julie missed home. Her friends, her simple routine, the heavy traffic on Bagley Road. Some nights she felt so lonely it hurt.

Most nights, after Dakota fell asleep, Julie jumped on the computer to update her blog on Dakota's Web site and read journal entries from friends. She appreciated their notes.

Still, she knew her friends could type something and go back to their normal lives with their healthy children. She had to get up the next morning and deal with Dakota.

Back at Duke, Julie loaded Dakota in her stroller and picked up her purse, so Ken and Bailey could catch their flight home. She was seething.

When they arrived at the airport, Dakota began crying.

Bailey cried, too, as she leaned over to hug Dakota. "How many days, Mommy?" Bailey asked Julie, holding her tightly around the waist.

"Five days, Bailey. Only five," Julie said. Ken would fly back to Duke so Julie could spend a week in Olmsted Falls with Bailey.

Ken kissed Julie and hugged her, long and tight. He picked up his bag and walked into the terminal.

Julie got back in her car and sobbed hard, gulping for air. She was so tired of being here. Tired of stuffing food into Dakota's mouth and force-feeding her medicine.

"Why won't you eat?" she yelled at Dakota, who was strapped in a booster chair in the back seat. Dakota stared at her mom, her head drooped to the side, a vacant smile on her face.

"Don't you know you're going to get a tube in your nose if you don't cooperate?" Julie said, sobbing.

Ken stared out the window of the plane. It was unusual for Julie to fly off the handle the way she did at the hospital.

His mind drifted, from the bills and the stack of Bailey's school papers on the kitchen counter, to where he parked his car this time and what he wanted for dinner. He knew Bailey would vote for KFC. He was sick of it. He was sick of commuting back and forth and being apart from Julie. He was sick of the whole Duke scene.

He and Craig Markowich, Alexis' father, had discussed funeral arrangements for their daughters. Just in case. Craig leaned toward cremation; Ken, toward burial. He thought Julie would need somewhere to visit, to talk to Dakota. Both fathers wanted to donate their girls' brains to Kurtzberg.

So often lately, when Ken's mind wandered, he would compose Dakota's eulogy in his head. He imagined himself standing before his friends and family, looking into their faces as he spoke.

When kids with these diseases die, we're the sad ones, but in a month, two months, six months, we'll be the lucky ones, because we knew them. We had the blessing of the way her smile lit up a room, of her personality, her sense of humor. All this crap, the blisters, the hallucinations, the puking blood, even with all this stuff, Dakota could still muscle out a smile. There's no way I could do that.

Ken leaned his head against the window and closed his eyes, fighting back tears. He had better keep Dakota's eulogy short. He didn't think he would make it through a long one.

Wednesday: A deep sense of loss.

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