

THE FIGHT TO SAVE ABBY & BELLE

Battling 'Childhood Alzheimer's'

Last year the Texas sisters were diagnosed with Niemann-Pick disease type C1—a rare genetic disorder sometimes called childhood Alzheimer's. Now the family is in a race against time to find a cure

BY NICOLE WEISENSEE EGAN



Abby (far left) and Belle “are happy girls,” says mom Pam (with her daughters at the doctor). “I see all they go through and I think, ‘If they can do it, I can do it too.’”



By the time a doctor recommended that Pam Andrews have genetic testing done on her 5-year-old daughter, she was already worried. Belle had met all her milestones, walking and crawling on time, but by age 3 she had started to trip a lot, twice falling so hard she broke her nose. Then her preschool teacher noticed her gait was a little off. Still, none of that could have prepared Pam and her husband, Chris, for the geneticist's devastating news: Their daughter had Niemann-Pick disease type C1, a rare and incurable progressive disorder sometimes referred to as childhood Alzheimer's because some of its symptoms—a gradual loss of speech and cognitive and physical abilities—mirror those of the brain disease. The disorder, doctors said, would likely claim Belle's life by age 15. “He said, ‘I encourage you to call Make-a-Wish and . . . go home and enjoy her,’” says Pam, tears spilling at the memory. “He said, ‘There's nothing we can do.’”

Two weeks later their heartbreak doubled: Belle's younger sister Abby, then 2, was also diagnosed with the disorder. Despite the bleak prognosis, the Austin, Texas, couple are now in the fight of their lives to save their daughters. In May 2016 they enrolled Belle in a clinical trial for a new drug to treat the genetic disorder and secured for Abby, who was too young to qualify for the trial, a compassionate-use exemption from the FDA to try the drug as well. Since then both girls have been on a regimen of biweekly injections that has so far shown promising results among partici-



HAIR & MAKEUP: LISA GLEESON/ZENOBIA

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Healing Help and Support 1. Abby (front) walks with her parents, Chris and Pam, Belle and Dr. Augustine Park, who administers the anesthesia during the girls' biweekly treatments at Dell. 2. Pam adjusts Belle's hearing aid. She started losing her hearing at age 4. "Belle wears them when she wants," says Pam. "They get hot." She also has trouble swallowing and wears plastic braces in her shoes to help correct her dystonia, which causes her feet and legs to turn inward. "It's heartbreaking to watch your kid struggle physically," says Pam. "There's just no other word for it."

The Bubble Room 3. Chris plays with Abby and Belle in their multisensory-experience room in their home. A friend donated the room's equipment (including the bubble-tube lamp behind Abby), which activates different regions of the brain to help slow neurodegenerative decline. 4. Belle does her at-home occupational therapy in the bubble room, but the girls love to play there too. They both get weekly physical therapy at home. Belle also has vision and speech therapy and, as of last September, a full-time aide at school to help her get around.

pants in a national clinical trial. And on Feb. 28 the Andrewses officially launched the Firefly Fund (firefly.fund), a nonprofit dedicated to finding a cure for NPC1 and other rare neurodegenerative genetic diseases that affect children. "There's no way I can sit back and just allow the two people who changed my life to be taken from me, not without a fight," says Chris, 45, an attorney. Adds Pam, 44, who works as a senior director at an international consulting firm: "We're going to do whatever it takes to find a cure for this disease and save our girls."

That unwavering commitment was in the cards from the day Belle was born in March 2010. "That was the exact moment everything in my life changed," says Chris, choking up as he remembers holding his 5-lb. 2-oz. baby daughter for the first time. "I said, 'The only thing I want to do is take care of this little girl.'" The couple, who had been living together for about four years when Pam got pregnant, decided to tie the knot that same year, with Chris cradling Belle in his arms as they both walked Pam down the aisle. Abby arrived four years later. She was two months premature and spent 60 days in the NICU before they finally brought her home. "Pam was there 16 hours a day,"

'WE DON'T LIVE WITH A BLACK CLOUD OVER US. WE LIVE WITH A LOT OF HOPE'

—PAM ANDREWS



Watch *People Features: The Andrewses Family*, available now on the new **People/Entertainment Weekly Network (PEN)**. Go to people.com/PEN, or download the PEN app on your favorite device.

says Chris. "I had to drag her out of the NICU every night."

By then Belle's symptoms had started to develop, turning their lives into a whirlwind of doctor's appointments (including visits with a pediatric orthopedist and a neurologist) and therapy sessions for speech, vision, motor skills and more. Test after test on Belle came back negative, but Pam couldn't shake the idea that something was terribly wrong. "I would lie awake and Google random symptoms on my phone," she says. "We were always going to some sort of specialist to try and figure out what the missing piece of this puzzle was."

It was Dr. Amy White, an Austin-based pediatrician, who started to put the pieces together in May 2015, when, during Belle's annual checkup, she noticed her swollen spleen—something doctors had consistently dismissed as nothing to worry about since Belle's birth. "She looked at me and said, 'How long has her spleen been like this?'" says Pam, starting to cry. "She didn't have to say anything more. It was the unspoken terror in her eyes." Dr. White recommended genetic testing and now says, "Things just weren't adding up. . . . I had suspicions on a broader spectrum but not NPC1 specifically, because I'd never seen it before. It's so rare."

Ten months later (it took six months just to get an appointment with a geneticist), the Andrewses listened in shock as doctors delivered Belle's dark prognosis. "We thought, 'This can't be happening to us,'" says Chris. "Chris was doing a lot of research every night," says Pam. "I was crying every night." They started exploring various treatment options and clinical trials, lining up trips to the National Institute of Health in Maryland and Rush University Medical Center in Chicago, where the girls initially started their biweekly-treatment regimen before switching to Ascension's Dell Children's Medical Center in Austin. Faced with medical bills and travel expenses totaling

as much as \$16,000 a month, Pam eventually lobbied the pharmaceutical company to move the treatments to Austin this past December. "We have nothing but gratitude," she says. "We would have done whatever it takes, but thankfully we didn't have to." By the time the girls began receiving their injections in late May, Chris was finding reason to hope, but Pam was still struggling. "We were not on the same page about where we thought our lives were headed," she says. "I thought we were headed to a cemetery."

That finally changed last June when the Andrewses attended a conference on NPC1 in Tucson. There they met other families battling the same disease and researchers who were working on a cure. "We laughed and cried with them," says Pam. "It was a pivotal moment in my life. I went from despair to . . . feeling there was a lot of hope." Chris adds, "I walked into a large room filled with scientists and said, 'Every single one of these people is trying to cure my daughter.' It made [the possibility of a cure] real for me."

In the meantime Abby and Belle are doing their part in the fight. Because Belle is enrolled in the clinical trial, the Andrewses don't know whether she's getting a placebo or the actual drug, called VTS-270. But when her yearlong involvement in the trial ends on May 20, she'll take the VTS-270. Meanwhile Abby has been receiving it all along—and has seen astonishing results. Prior to the treatment, she was still on the bottle and hadn't been potty trained, says Pam. "Now she's taken off like a rocket. She's talking a lot. She started preschool. She's doing gymnastics. It's like someone cleaned the cobwebs out for Abby."

As encouraged as they are by Abby's progress—and the hope it inspires for Belle—the Andrewses have learned to live in the moment, as their daughters do. They love arts and crafts, swimming and playing with their two beloved dogs, Billy and Leo. "I have to meet them every day where they are," says Pam. "They laugh a lot. They play. They giggle. I believe with every fiber in my body that my girls are going to live and they are going to be happy." ●

Day by Day
"Even though it's such a hard thing to live positively, you have to do that," says Chris (at home in Austin with Pam and the girls). "Because you do your children no service by moping and weeping. You have to live for now."

