Salem family raises $900K to battle rare Batten disease

Capi Lynn
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Melissa, Haley, Dean, Cole, Maddie, Audrey and their dogs Gary and Lucy at their home in Salem on Saturday, Feb. 23, 2019. MICHAELA ROMÁN / STATESMAN JOURNAL

Haley Pollman loves animals, Barbies, princess dresses and unicorns, not necessarily in that order.

Visit her South Salem home and you're likely to meet the furry four-legged members of the family before parents or siblings.

Haley does the introductions on the porch, stroking each animal to make sure she's naming the right one, Fred and Ginger the pygmy goats, and Gary and Lucy the dogs.

She's lost her sight, a symptom of a rare and deadly genetic disorder called Batten disease, but it doesn't stop the 9-year-old from riding her pink jeep in the yard or her purple moped on the driveway. It doesn't stop her from exploring the rest of the property, which she's proud to say has a secret garden and a secret playhouse. And it sure doesn't stop her from snow skiing down a mountain or water skiing on a lake.

"Haley is one of the bravest people I've ever met," her dad Dean says.

Mom Melissa and older siblings Audrey, Maddie and Cole agree.

"She's active, she's happy, and she's social," Melissa says. "We try to take our lead from that."

Batten disease affects the brain and nervous system. Symptoms sometimes begin in infancy, sometimes later in childhood, and it's almost always fatal.

But there is hope. The Pollmans have connected with a lab in South Dakota where some of the best scientists in the field are doing promising research on gene therapy, and they're raising money for an FDA-approved clinical trial.

"We feel they're on the brink of finding a cure for this thing," Dean says. "We're trying to do the best we can to make a difference. We just know we have to do it quickly."

They launched Haley's Heroes Foundation early last year and raised more than $900,000. Their goal this year is to raise at least that much. Foundation merchandise, including T-shirts, sweatshirts, hats, even bottles of wine, are emblazoned with unicorns, a symbol of strength, hope and healing power and one of Haley's favorite things.

Her world remains a happy place filled with glitter, magic and all things purple and pink.

Symptoms start with vision loss

Between the first and second grade, Haley began experiencing vision problems. She started pulling things close to her face and struggled to read.

She was prescribed glasses, but her eyesight continued to deteriorate.

Her parents spent the better part of a year taking her to various specialists. They eventually were referred to Oregon Health & Science University, where the results of a genetic test forever changed their world.

"These children are 100 percent healthy — until they're not," Melissa says. Haley was diagnosed with Batten disease in October 2017. The prognosis included three words no parent ever wants to hear: Rare. Fatal. Incurable.

There are 13 forms of the neurodegenerative disease — also known as ceroid lipofuscinosis, neuronal (CLN) — and each is given a different number designation. Haley has CLN1. Her parents have been told there are about 1,000 documented cases worldwide, although some believe that would be on the high side. Experts estimate all forms of Batten disease affect around 1 in 100,000 births worldwide.

The disease varies in each child, although there is overlap no matter what form. Early symptoms usually appear between the ages of 5 and 10 years old and children affected generally are rendered blind, immobile and cognitively impaired. Children with Batten disease often die by their late teens. Dean and Melissa didn't procrastinate or hold back delivering the news to Haley's siblings. The last thing they wanted was for them to Google the disease before hearing about it from them first. Batten disease is as difficult to diagnose as it is to explain. It's a family of rare disorders caused by genetic mutations that affect cells in the brain and lead to devastating and irreversible effects.

Lysosomes in the cells don't function properly, failing to break down proteins and dispose of other waste.

Melissa Pollman describes it this way: If the garbage collector stopped taking away the garbage, everything might be OK for a while but eventually, the garbage would overflow and rot everything around it. Haley doesn't have a garbage collector.

Mom on a mission to save her child

Melissa is gathering as much information as she can about Batten disease.

She recently attended a scientific symposium on lysosomal disease research in Orlando, Florida. Batten is a lysosomal storage disease. She and Dean quickly learned how important connections are when it comes to fighting for treatment for a child with a rare disease.

She stepped away from the family construction business last year to become a full-time patient advocate and fundraiser. She's focusing on raising money and meeting with researchers, pharmaceutical companies, and other family foundations.

They're connecting with other families through the Batten Disease Support and Research Association, which funds and facilitates scientific research throughout the drug development process. Melissa and Dean attended a conference last summer.

They've been to the Batten Disease Center of Excellence at the University of Rochester Medical Center in New York, most recently in December after the family went on a Make-A-Wish trip for Haley to Disney World.

The center provides support and clinical services and works to find treatments that will slow or stop the progression of the disease.

"With the right doctors, there's a lot of research out there," Melissa says. "There's a lot of hope."

They know of two foundations that have successfully funded gene therapy clinical trials for children with other forms of Batten disease.

"If anybody's going to pay attention to it, someone's got to start the research, and who better than the families?" Melissa says. "You're not going to get more passion than from a parent trying to save their child."

Small world, even in rare diseases

Kristyn Lara can relate. Her son Andrew, 7, also has Batten disease.

Andrew is a happy, caring boy who loves cars, trucks and construction vehicles, basically anything that goes.

He was diagnosed in January 2016, has CLN3, and lives just a couple miles away from Haley.

Their moms discovered they were practically neighbors after meeting online in an online support group for Batten parents.

"It would be really nice to meet in person," Kristyn said. "We're both going through the same journey."

The online support group is a blessing for Kristyn because husband Rick works out of state.

"It's just nice for people to understand what we're facing and when you have one of those days where you're frustrated, they're there to help you through it," she said.

The disease has progressed rapidly in Andrew, who attends Battle Creek Elementary School. He's lost his sight, too, uses a wheelchair much of the time, and recently had surgery because of difficulty swallowing and eating.

A feeding tube now delivers nutrition directly to his stomach.

The Laras don't have a foundation, but they're fighting just the same to save their son. Hearing what the Pollmans are accomplishing gives them hope, too.

Gene therapy studies on mice

Jill Weimer is one of the experts on Batten disease. She's the senior director of therapeutic development at Sanford Research in Sioux Falls, South Dakota.

Her lab receives funding from Haley's Heroes Foundation and is working on projects for six different variances, including the one Haley has.

"The biggest hope right now is gene therapy," Weimer says. "You take the common cold virus, strip out the bad parts, and trick the virus into carrying a healthy copy of CLN1 into cells."

Her lab is testing how safe and effective gene therapy is on mice with CLN1.

Developing a therapy or drug can involve years of experiments on animals and human cells before it ever gets to the clinical trial stage.

How soon could a clinical trial be possible for Haley's form of Batten disease?

"I would hope and pray 6 months to a year," Weimer says. "But a lot of things are out of my control.

"The families don't have the luxury of time. One of the things our lab has really focused on the last three years is coming up with ways to do the science faster."

Batten researchers compete for research dollars against counterparts working with diabetes, cancer and other widespread illnesses, and it's often a number's game.

That's why private foundations raising money for rare diseases are so critical.

Weimer confirms funding from two foundations brought gene therapy to clinical trial for their child's form of Batten disease.

"That's inspiration for the Pollmans," she says. "Like those families, they didn't take no for an answer. They are knocking on every door looking for hope for their daughter."

Weimer stays in regular contact with families who help fund her research, including the Pollmans.

"Just know how important the families and the foundations are in this journey," she tells the Statesman Journal. "I learn a lot from them about the disease and the patients."

She has yet to meet Haley, although she's working with the girl's pediatrician at Kaiser Permanente to procure a sample of her cells.

"We take a tiny punch of her skin about the size of the tip of a pin, bust apart those cells, called fibroblast cells, grow them in a dish and keep them forever in my lab for drug screening," Weimer says. "We can see how drugs affect the cells."

Community support beyond Salem

Haley attends Montessori Discovery Center and Lee Elementary School.

A fundraiser at Lee recently raised $1,700, and students celebrated Feb. 28 by dressing up as zebras or unicorns — zebras to represent for Rare Disease, unicorns for Haley's Heroes Foundation.

Haley uses a cane at school, although she prefers not to. She doesn't like the word blind, either. She just sees differently than everybody else.

While at Disney World, her siblings helped her visual the rides by describing what they saw. Her favorites were Soarin' Around the World and Avatar Flight of Passage, both flight simulators that tap into senses other than just sight.

Haley gets frustrated she can no longer read, and it was upsetting not being able to see fireworks last summer.

"She's a typical 9-year-old," her mom says. "She doesn't want to be different."

She doesn't see herself as different, either. When Salem Academy did a coin drive to benefit the foundation, the school invited Haley to an assembly and after being introduced, corrected the principal.

"I'm not sick," Haley said. "I just have Batten disease."

While in Florida on the Make-A-Wish trip, she asked her mom and dad if people die from Batten disease.

"That was hard," Melissa says. "She looked at me and said, 'I don't want to die.' I told her, 'Dad and I are doing everything we can to fight'

"Fifteen minutes later — kids are so resilient — she's like, 'I have a perfect life' while Dean and I are a ball of mush trying to pick ourselves up."

The goal for Haley's Heroes Foundation is to raise $1 million this year and meet with a handful of key foundations across the country that have been at this much longer.

The hope is to pool their resources and share expenses, such as lab overhead.

"Ultimately, we all have the same goal, to bring awareness and find a cure," Melissa says.

Promising research is underway, but sadly it's not happening fast enough for many Batten families.

"We're really trying to push the science forward," Melissa says. "There are researchers out there who want to do this, but it's all about the money."

How to help

Visit haleysheroesfoundation.org to donate or learn more about the foundation and upcoming fundraisers including "Hops for Hope" on May 18 at b2 Taphouse & Brewery and the "Bid to Rid Batten Auction" on Sept. 14 at Salem Convention Center.

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