A Stem Cell Transplant Restores a Little Girl’s Future

A Deserving 9-Year-Old Ushers In Two Firsts

3-D Printing Helps With Complex Heart Repairs
SPRING 2016
A newsletter for friends of Duke Children’s

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Letter from the CHAIR

Dear Friend of Duke Children’s,

Creating a warm, family-friendly atmosphere has always been a core value of Duke Children’s. The new Pediatric Cardiac Intensive Care Unit (PCICU) is a shining example of that commitment. This new unit, opened in November 2015, not only features the very latest technology, but just as important, more space and comfort for families. In this issue, read about Shania Harris, the first patient to move into the new PCICU, after being the first to receive a heart-plus-other-organ transplant at Duke Children’s.

The beautiful face on the cover is Charlee Rae Gribble. You can read about her family’s journey through a stem cell transplant after being diagnosed with a potentially fatal genetic disorder. And don’t miss the story about 3-D printing and how this technology is helping pediatric cardiologists repair heart defects in a way that is truly individualized.

I loved reading these stories of courage and progress, and I hope you do, too.

Because nothing matters more,

ANN M. REED, MD
Chair, Department of Pediatrics

HOW YOU CAN HELP

You can support lifesaving research and patient care at Duke Children’s. Make a gift any of these ways:

1. Use the envelope bound into this newsletter
2. Use the secure online form at bit.ly/SpringStories
3. Call us at 919-385-3138
DUKE MEDICINE BECOMES DUKE HEALTH

In January 2016, Duke’s Chancellor for Health Affairs A. Eugene Washington, MD, announced that Duke Medicine has been renamed Duke Health, reflecting the overarching aspiration of improving health.

“Duke Health signals our intention to explore more comprehensive approaches to health that extend beyond medical care and into other determinants of population health improvement,” wrote Washington. “And Duke Health represents a more inclusive and synergistic approach to maximizing contributions to health improvement from the diverse array of disciplines and schools that comprise Duke University, as well as from our external partners.”

Nurse honored for work in Kenya

Jessica Thomsen, Duke Children’s nurse, won the Dr. Martin Luther King Jr. Community Caregiver Award for starting Jemo House in 2012 in Maseno, Kenya, to provide a safe, loving home for kids whose lives have been impacted by HIV.
Three Duke Children’s faculty members, Ricki F. Goldstein, MD, (neonatology); Laura E. Schanberg, MD, (rheumatology); and William J. Steinbach MD, (molecular genetics and microbiology), were recently elected to the American Pediatric Society (APS).

In addition, five faculty members, Christoph P. Hornik, MD, MPH, (critical care medicine); William B. Gallentine, DO, (neurology); Sarah C. Armstrong, MD, (primary care); Kevin M. Watt, MD, (critical care medicine); and Pinar Gumus Balikcioglu, MD, (endocrinology) were elected to the Society for Pediatric Research (SPR).

According to Ann Reed, MD, chair of the Department of Pediatrics, “Election to the APS/SPR represents a true distinction, and these faculty members are highly deserving of this recognition.”
New Therapy for Pompe Disease Developed

Researchers at Duke have developed another therapy for Pompe disease, a condition that used to take infants before their first birthday.

Duke research led to the first effective treatment for the disease, a drug called Myozyme. Development of the drug was the subject of a 2010 movie called Extraordinary Measures.

Despite treatment, some patients still have trouble breathing. The newest therapy helps patients breathe easier. A study led by Duke speech pathologist Harrison Jones, PhD, tested a regular regimen of respiratory muscle workouts using small devices. Patients in the study showed strength increases in their breathing muscles during a 12-week period, and they maintained those increases three months after stopping the regimen.

DUKE CHILDREN’S LAUNCHES PSYCH EVALUATION CENTER

Mental illness affects approximately one in five children, but nearly 80 percent of those affected do not receive any treatment. Many families find it difficult to access care, or face lengthy waitlists when they do. To address this issue, leaders from Duke Children’s, the Department of Psychiatry and Behavioral Sciences, and the Department of Pediatrics collaborated to launch the Duke Children’s Evaluation Center (DCEC) in fall 2015.

The DCEC offers a welcoming and family-centered environment for children and families who are seeking mental and behavioral health services. An interdisciplinary team of social workers, child psychologists, and child psychiatrists provide evidence-based assessment and treatment for infants, children, adolescents, young adults, and families. The center can also provide referral coordination for families seeking long-term mental health services, as well as prompt follow-up care for children who have been seen for psychiatric care in the Duke Children’s Emergency Department.

The clinic is open 8 a.m. through 5 p.m. Monday through Friday; call 919-385-3232 for referrals.

Radiothon Is Coming In September

The MIX 101.5 Radiothon for Duke Children’s will be September 7-8, 2016. This annual event is one of Duke Children’s most visible and most successful, with 100 percent of proceeds going to Duke Children’s.
A DESERVING 9-YEAR-OLD
USHERS IN
TWO FIRSTS

BY DAVE HART
SHANIA HARRIS, 9, SITS ON AN EXAMINATION TABLE IN A CLINIC ROOM AT THE DUKE CHILDREN’S CARDIOLOGY UNIT. She’s wearing a pretty red dress and happily making her way through a bag of chili-cheese flavored chips, smiling shyly and teasing her great-uncle, Tony Jenkins, who she calls “Papa,” about how much better she did than he last Christmas.

“Papa didn’t get any toys,” she says. Papa can’t do much but laugh and acknowledge that, yes, Santa didn’t bring him any toys.

But he and the rest of Shania’s large family got something much more precious last holiday season: they got Shania back.

The best present
After spending almost a year in the hospital—a year of endless rounds of tests and treatments for potentially fatal kidney and heart conditions, a year of sleepless nights for her mother and other family members, and a year in which she became the recipient of Duke Children’s first-ever heart-plus-other-organ transplant—Shania finally was discharged on December 11, 2015. She had been in the hospital, mostly in the Pediatric Cardiac Intensive Care Unit (PCICU), for 322 days.

“That was the best Christmas present ever, having Shania come home,” says Shania’s mother, Ashley Robinson. “It was a long, scary year, and we were so happy to have her home and start getting our lives back to normal.”

Heather Henderson, MD, Shania’s lead cardiologist, said her family played a key role in her treatment and recovery.

“She’s back home and doing great,” says Henderson. “She has a huge family; we celebrated her birthday over the summer in the lobby of Duke Children’s, and there must have been a hundred people. It’s an amazing family support network, and that really helped her get through all this and keep her on track. I think they probably fight over who gets to care for her and bring her to her appointments.”

Bad news, then worse
Shania fell ill in January 2015, complaining of feeling poorly and having trouble breathing. Her mother took her to Duke Regional Pediatrics, where doctors quickly determined that she needed a comprehensive evaluation and had her transferred to Duke Children’s Emergency Department.

Doctors at Duke found that her kidneys weren’t functioning properly, and neither was her heart. Shania had had surgery as an infant to repair a hole in her heart, but she had recovered fully from that and had been enjoying a healthy childhood until she became ill in early 2015.

After thorough testing, Duke doctors put her on dialysis to improve her kidney function and began treating her with medications to help her heart.
“Sometimes when you treat kidney failure and get rid of the toxins that build up when kidneys don’t work well, the heart also starts to recover,” says Henderson. “That’s what we hoped would happen. We gave her a month or two, but despite adequate dialysis, her heart wasn’t recovering any function and she was still depending on IV medications to compensate. We tried a few times to scale those medications back and wean her off them, and her body didn’t tolerate that at all. At that point, we started to talk about the possibility of a heart transplant.”

On waiting lists
The pediatric kidney team was already recommending a kidney transplant. Duke Children’s had never before performed a simultaneous transplant of a heart and another organ, but Shania’s case was unique, and she was put on waiting lists for both heart and kidney.

In the meantime, she lived in the PCICU, where she had her stuffed animals and pictures on the walls and was cared for not only by her pediatric team but by her family. Her bubbly spirit infected the whole place.

“Can I call her a diva?” says Henderson. “She loves to have her fingernails painted, and if you let her, she’ll paint yours. She loves singing and dancing; she wanted to make a YouTube video, and she talked all of us doctors and nurses into singing and dancing and wearing props.”

In November 2015, a suitable heart and kidney from a single donor became available, and her team prepped her for surgery. Early on the morning of Nov. 18, the heart transplant team replaced her failing heart with the new one. The surgery went well, and she was taken back to the ICU for several hours.

“With her vital signs like a hawk,” says Henderson. “She did great. No complications, no major hiccups, and at about 3:00 p.m. she went back in for the kidney transplant. As soon as they put the kidney in, it started to function. Everything went beautifully.”

A room with a view
When Shania woke up, she was in a different room—an entirely different unit, in fact—than the one she’d spent the
bulk of the past year in. The old PCICU has been replaced with a new PCICU one floor down, and by happenstance the new one opened within a day of Shania’s surgeries. She and the other current patients who were old enough had been given a tour of the new unit and invited to choose their own rooms; Shania, as the child who had been there longest, got first pick and chose a corner room with two windows.

The new unit has upgraded monitors and equipment, more space for staff and visitors, better access, and, not least, individual rather than shared patient rooms.

“Everybody has a lot more room,” says Henderson. “It makes a difference, especially if you’re a patient who’s in there for a long time. It’s hard enough being in an ICU for 320 days. We want to make everybody as comfortable as possible.”

Home at last

Shania recovered rapidly. With her new organs working well, doctors were able to draw down her medications and take her off dialysis. Just a shade over three weeks after undergoing a double organ transplant—and more than 10 months after entering the hospital—Shania was wheeled out the front door of Duke Children’s to go home in a limousine driven by a Christmas elf, courtesy of an aunt who had made that promise to her.

She will return to Duke for follow-up visits, but if she continues to do well those trips will become less frequent over time. She’s doing her schoolwork at home now, helped by Duke’s homebound services staff, but eventually she’ll transition back to school.

“The doctors, nurses, and staff at Duke were absolutely wonderful,” says Beverly Jenkins, Shania’s great-aunt and, along with Shania’s mother, one of her two primary caregivers. “They were there at every step of the way for Shania. They made us feel welcome, made us feel comfortable, and took the time to explain everything. I don’t have words to express how well they treated us.”

“He loves to have her fingernails painted, and if you let her, she’ll paint yours. She wanted to make a YouTube video, and she talked all of us doctors and nurses into singing and dancing and wearing props.”

— HEATHER HENDERSON, MD
Pediatric Cardiologist

How to make a gift to Duke Children’s

You can support more families like Shania’s. Go to bit.ly/SpringStories or use the envelope bound into this newsletter. Thank you!
THE SHAPE OF THINGS
3-D PRINTING IS HELPING DOCTORS PLAN FOR COMPLEX REPAIRS OF LITTLE HEARTS
BY ANGELA SPIVEY
“[3-D printing] improves our ability to diagnose and care for children and lets us provide truly individualized medicine that is unique to that child.” – PIERS BARKER, MD

Pediatric Cardiologist
PIERS BARKER, MD, HOLDS A RED, FLEXIBLE PLASTIC HEART, turning it over in his hands. It’s an exact replica of a particular child’s heart, printed on a 3-D printer using information from MRIs and CT scans. The child has several heart defects, including a ventricular septal defect—basically a hole between the heart’s two pumping chambers.

Barker pushes and prods the plastic, examining the defect from every angle. How does the blood flow out of the heart and into the lungs? Will sewing a patch over the hole hinder that flow? Will surgeons need to reroute it?

Until now, the best he could do was look at an image of a child’s heart on a computer screen. Collaborating with Tawfiq Khoury, MD, a resident in the Duke Department of Surgery, Barker worked with engineer Chip Bobbert in Duke’s Innovation Studio to print heart models to help plan repairs for two children with very complicated defects.

For this particular child, the model helped Barker and a team of colleagues decide to wait until the child was a bit older and the heart had grown more to correct the defect.

“After 20 years in pediatric echocardiography, nothing has helped me more than a 3-D model to understand the defect and understand what is best to do with each patient,” Barker says. “Being able to pass the model back and forth helps us make sure everyone on the team has the same mental picture.

“It improves our ability to diagnose and care for children and lets us provide truly individualized medicine that is unique to that child,” he says.

Their collaboration has led to Duke University Hospital launching a 3-D Printing Task Force to plan how to use the technology on a larger scale. The group is deciding what type of printing to invest in, which conditions will benefit most from the modeling, and how to ensure that privacy rules and other rules are followed.

Barker envisions that in the next decade, images such as CT scans and MRIs will be only the first step in evaluating and planning treatment, as 3-D printing becomes more practical for more patients. “Technology has advanced to make this easy to do,” he says.

Pictured below: Tawfiq Khoury, MD, Chip Bobbert, and Piers Barker, MD,

New and better ways of treating young patients requires innovation like this. You can support Duke Children’s research. Use the envelope in this newsletter or give online at bit.ly/SpringStories.
WHEN CHARLEE RAE GRIBBLE OF SPARTANBURG, SOUTH CAROLINA, WAS ONLY A FEW MONTHS OLD, her parents noticed troubling symptoms, including terrible nasal congestion, a rounded spine, and a flat nasal bridge. They visited pediatricians, orthopaedic specialists, and geneticists in search of answers.

Charlee Rae was diagnosed with Hurler syndrome, a rare genetic disorder. The family was referred to Duke Children’s, where they met with Vinod K. Prasad, MD.

“He told us that Charlee Rae had the severe form of the condition, and if not treated, her life expectancy would be less than 10 years,” says Jessica Gribble, Charlee Rae’s mother. “My husband and I lost it. I was holding Charlee, and I thought, I don’t want 10 years. I want more than that.”

A difficult journey

Prasad explained to Jessica and her husband, Russ, that to give Charlee Rae the best chance at a long and healthy life, they should consider a cord blood transplant. It would be a difficult journey with risks and complications.

The Gribbles seized the opportunity for this innovative therapy and began making major life changes in order to prepare. Jessica, a middle school science teacher, asked her principal for a year off to care for Charlee Rae and her other daughter, four-year-old Mali, and her request was granted without hesitation.

A stem cell match was found for Charlee Rae from donor cord blood, through Carolinas Cord Blood Bank, a not-for-profit public bank that is part of Duke. The family was offered an apartment near Duke at no expense to them, to live in during Charlee’s treatment. The apartment was sponsored by the HJI Foundation (Hayden’s Journey of Inspiration), started by a family whose daughter had also received a stem cell transplant at Duke.

To prepare her body to accept the new stem cells, Charlee Rae endured a high-dose treatment of chemotherapy that wiped out her immune system. She also had surgery to remove her tonsils and took powerful immunosuppressive medication to help prevent graft-versus-host disease, a complication of transplantation. She qualified for a clinical
trial at Duke, which was testing the effects of adding prostaglandins to stem cells to speed up the process of her body accepting the donor cells.

“And then we braced ourselves,” says Jessica. “It was hard because there was nothing else we could do, but she was in God’s hands and at the best medical facilities. I just asked the doctors, ‘Save her.’”

A life-saving syringe of cells

On July 16, 2015, Charlee Rae was ready to receive her new stem cells. “They brought in a syringe of donor cells and I said, ‘Is that it?’ I thought it would be a big bag,” says Jessica.

“The research group was sitting outside the room, taking notes. Charlee slept through the whole thing. In an hour, we were done.”

When Charlee woke up, she was happy and full of energy, says her mom. “I asked them what they put in that syringe!” laughs Jessica.

In just two weeks the cells had grafted – an unusually quick engraftment. Charlee was on her way.

In the weeks that followed, the Gribbles watched Charlee’s white blood count go up and down. She was tired and battled fevers and side effects of the chemotherapy, including pain and hair loss. “She started on her way back up after seven weeks,” says Jessica.
“It was hard because there was nothing else we could do, but she was in God’s hands and at the best medical facilities. I just asked the doctors, ‘Save her.’”

— JESSICA GRIBBLE

After months in their home-away-from-home, Prasad told the family it was time to go to their real home, for good. “Charlee Rae’s recovery after transplant was very quick and her resilience is amazing,” says Prasad. “I was truly thrilled how quickly she engrafted and how quickly her immune system recovered, and she had no graft-versus-host-disease. Transplantation has opened doors to a long life for many children with rare and fatal diseases.”

Full of surprises

Today, two-year-old Charlee Rae is healthy and happy. Jessica says her daughter’s future physical development might not be on par with her peers, but her intellectual development is right where it should be. In fact, Charlee Rae is full of surprises.

Charlee Rae is learning sign language by watching her parents, who are both deaf, and even makes up her own signs to communicate her wants and needs. “When I give her Pediasure,” explains Jessica, “I shake the bottle first. So now when Charlee wants her Pediasure, she makes a shaking motion with her hand. She’s very smart. I think she’ll be just fine.”

WANT TO HELP?

You can support the innovative care that Duke Children’s is known for. Use the envelope in this newsletter, or give online at bit.ly/SpringStories.
1. **Gaming for Duke Children’s**

   Video and board game enthusiasts raised over $91,000 for Duke Children’s by participating in Extra Life, a Children’s Miracle Network Hospitals fundraiser that took place in November 2015.

   Extra Life lets gamers do what they love — play games — while seeking donations from friends and family for the 24-hour fundraiser. Some participants played from home while others joined fellow gamers at the Frontier in Research Triangle Park. To learn more about future events, visit Extra-Life.org.

2. **NC Comicon Chooses Duke Children’s**

   NC Comicon, held November 11-13, 2015, made a gift of $15,703 to Duke Children’s, their chosen charity for the event. The event had a record-breaking crowd of over 10,000 attendees at the Durham Convention Center. Special guests included Gerard Way, comic book writer and lead singer of the band *My Chemical Romance*; and *The Walking Dead* artist and co-creator Charlie Adlard.

   Proceeds from sales of autographs by Way and Adlard comprised the donation to Duke Children’s. The convention featured more than 200 exhibitors and a cosplay prom party, Guardians of the Gala.

3. **A Walk to the Coast**

   Buxton Ward, 17, and his mother, Paisley Ward, walked from New Bern to Atlantic Beach, North Carolina (45 miles) to raise funds for Duke Children’s. The pair made the fundraising trek in appreciation of care received from Robert Fitch, MD, pediatric orthopaedic surgeon.

4. **Speedway Gets a Speedy Start**

   Gasoline and convenience store chain Speedway raised over $430,000 for Duke Children’s in its first year of fundraising for Children’s Miracle Network Hospitals. Welcome aboard, Speedway, you’re amazing!
BOUNCING ALONG

5 Dancing for the Kids
At dance marathons at Duke University, NC State University, and Elon University, students dance for hours, raising money along the way for Duke Children’s. Students at Duke University hosted the third annual Duke Dance Marathon in November 2015, raising $101,931. NC State completed its fourth annual Dance Marathon (pictured) in February 2016, raising $80,312. Both schools experienced their highest fundraising totals to date! ELONTHON at Elon University rounded out the dance marathon event season with its 24-hour marathon in April.

6 A Short Stack with a Purpose
Miss Garner 2016, Grace Ann Carroll, and her family enjoyed free pancakes at their local IHOP on National Pancake Day, March 8, 2016. The annual fundraiser benefitting Children’s Miracle Network Hospitals brings in pancake lovers from across the nation, who are encouraged to make a donation to their local CMN hospital.

This year, Miss North Carolina local title holders and Kiwanis/Key Club members volunteered their time at local IHOPs to make the day an even bigger success. Restaurants in Duke Children’s fundraising area (the Triangle, the Triad, and Wilmington) raised over $70,000 for Duke Children’s from their balloon sales in February and National Pancake Day.

7 Portrait of a Volunteer
As a longtime employee of Sam’s Club in Goldsboro, Vicki Mozingo had always participated in the store’s yearly campaign to raise money for Children’s Miracle Network Hospitals. Six years ago, she made a decision that she could do more.

“One day, before my husband passed away, we decided we wanted to do something for children,” says Vicki. “We never had any children of our own. I decided not just to raise money during our campaign, May through June, but all year long.”

By her own accounting in her own notebook, Vicki estimates she has personally raised over $63,000 since 2010.

“Each day I say, ‘God bless the children today so they can get the help they need at Duke,’” she says. And folks respond. “People look at your heart and know you’re genuine;” she says. “Some people make a donation every time they come in the store.” Vicki says she makes sure donors know the money raised at the store stays at Duke Children’s.

At a recent Children’s Miracle Network Hospitals meeting of corporate partners, associates, hospital representatives, and champion patients in Florida, Vicki had the pleasure of meeting Marie Osmond, co-founder of Children’s Miracle Network Hospitals.
YOU CAN BE A DUKE CHILDREN’S COMMUNITY PARTNER

Want to host your own fundraising event for Duke Children’s? We love that idea!

For information, please contact Amelia Howle at amelia.howle@duke.edu or 919-385-0033
SAVE THE DATE

Duke Children’s GALA

SUNDAY, OCTOBER 22, 2016 • WASHINGTON DUKE INN & GOLF CLUB

A premier fundraising event to support Duke Children’s.

For information: dukechildrens.org/giving/events/gala