



St. Jude promise

stjude.org/promise autumn 2018

Education
and **hope** for
families with
rare **genetic**
syndrome *page 16*

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St. Jude Global
saves young lives

Boosting survivors'
problem-solving
skills and **memory**



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The mission of St. Jude Children's Research Hospital is to advance cures, and means of prevention, for pediatric catastrophic diseases through research and treatment.

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A Big Heart for St. Jude

One donor honors her children by supporting the kids of St. Jude.

By Kerry Healy

Natalie Charach (right) and her St. Jude representative, Erika Rudd

NATALIE CHARACH has a big personality and a heart to match.

"I am 92 years old, and I'm going to speak my piece," she declares.

This lively lady has great compassion for the patients and families of St. Jude Children's Research Hospital. With the help of her St. Jude representative, Erika Rudd, Natalie recently found a way to honor her son and late daughter by naming a playroom in an inpatient unit of the Kay Research and Care Center at St. Jude.

"I thank God I was able to do that," Natalie says. "I know my support is going to help the patients and the research, and I'm very proud of that."

Natalie's late husband, Manny, helped introduce Japanese consumer goods to Americans by helping rebrand the Matsushita Electric Industrial Co. into Panasonic, now a worldwide leader in consumer electronics.

The couple included St. Jude in their long-range financial plans. But after Manny's death, Natalie made a slight change in timing. There is a Yiddish word she

recalls that perfectly captures her feelings.

"I wanted to give now because I wanted the *nachas* (pleasure and satisfaction) of seeing the good St. Jude did for the children," she says.

The memory of helping someone in need fuels Natalie's passion for supporting St. Jude patients and families.

When her daughter, Janice, was battling cancer as an adult, Natalie encountered a man in the hospital whose wife was dying from cancer. Natalie was able to provide assistance. The impact of that support is forever etched in her memory.

"There was so much sadness in his life and on top of that — financial problems," Natalie says. "I was glad I could help."

Sadly, cancer claimed Janice's life a year later at the age of 38. Natalie's son, Jeff, is a cancer survivor.

"Both my children, being talented artists, loved color and play," Natalie says. "My son loves children, and he is so happy that I named the playroom in honor of him and his sister."

In supporting St. Jude, Natalie has found kindred spirits.

"Everyone cares there. I feel like I am part of a family," she says. "You are never alone when you are part of St. Jude." ■

Learn how to include St. Jude in your long-term financial plans:

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EXERCISE your options

By Elizabeth Jane Walker

A fun fitness study helps children increase activity after leukemia treatment.

Every evening, parents and kids across the nation sprawl across couches, staring at TVs or other electronic devices. But in the Davis household, 12-year-old Travis is likely joining his dad in a regimen of stretches, crunches and planks. It's one way the seventh-grader can earn activity points in an exercise study for survivors of acute lymphoblastic leukemia (ALL).

Travis is enrolled in a clinical trial headed by Kiri Ness, PhD, of St. Jude Children's Research Hospital. The study aims to enroll 384 children and teens in more than 60 sites around the world. Ness and her colleagues are exploring whether a reward-based, interactive website can improve the fitness, quality of life and school attendance of ALL survivors between the ages of 8 and 15.

“During therapy, patients often experience cancer-related fatigue,” says Ness, a faculty member in St. Jude Epidemiology and Cancer Control. “Patients are out of commission for a while during chemotherapy, and once they become sedentary it’s more difficult to regain their strength and energy.”

“We’re hoping the kids who participate in this program will become more physically active. That would have positive implications for their long-term health.”

The risks of inactivity

Ness recently joined forces with St. Jude oncologist Hiroto Inaba, MD, PhD; Emily Browne, DNP, RN, Transition Program director, and other colleagues on a separate study that measured the body mass of children with ALL. The team found that most children put on weight during treatment, and the weight gain often continued afterward.

“Today, we have excellent survival rates for ALL, but we need to be careful about adverse effects – both short- and long-term,” Inaba says. “The percentage of overweight and obese patients in our study increased from approximately 25 percent at diagnosis to 50 percent during the off-therapy period. At the same time, their height growth is affected, especially in patients who are 10 years or older at diagnosis and in those with other risk factors. We’re taking steps to develop supportive-care efforts for these children.”

By intervening early, researchers hope to help young ALL survivors maintain a healthy weight – while establishing good habits that will last a lifetime.

Hop, skip and jump

Each child in the two-year clinical trial wears a physical activity monitor that resembles a wristwatch. The child regularly uploads data from that device into an interactive website.

Participants randomly assigned to the study’s control group receive educational instruction and limited website access. They can view their progress and earn rewards based on points they accrue through physical activity.

The second group gets full access to the website, where they view the progress of their peers, interact with them and earn rewards.

“We want to know whether social interaction and competition increase their physical activity,” explains study coordinator Sarah Terrell. “Does it take peer pressure as well as rewards to motivate these kids to become more active?”

Children in the study receive periodic blood tests, body measurements and fitness evaluations. They also report on their general health, school attendance and quality of life.

Participants can earn points for any type of physical activity, whether it’s walking the dog, stretching before bedtime or helping their parents prepare meals.

“As part of our education component, we also instruct the patients on modifications they may need to make in order to

be active,” explains Jennifer Fournier, who handles the study’s collaborating sites.

Inherent rewards

Travis has been excited to set new goals and earn movement points.

“He knew he needed to be active to build the strength and endurance he’d lost during treatment,” his mom says. “This study has given him the opportunity to see how much he’s moving, and it has motivated him to get up and do more.”

Recently, Travis set his sights on learning to play the guitar and speak French. But he also has a third aspiration: He wants to play baseball, a sport he enjoyed before his illness. Through the St. Jude clinical trial, Travis has already rounded third base – having built energy, strength and resilience.

He anticipates that soon he’ll be sliding into home plate.■

“We’re hoping the kids who participate in this program will become more physically active. That would have positive implications for their long-term health.”

– Kiri Ness, PhD



Check-up, check in

Facing page: Travis Davis completes a physical assessment with Physical Therapist Lindsey Christoffersen. Above: “This study provides a nice way to insert physical activity into the lives of children today, who are attached to the Internet,” says Jasmine Hewlett, who assists Travis with a survey.



Where Hope is Reborn

THANKS TO ST. JUDE GLOBAL'S WORK IN JORDAN, MORE CHILDREN WITH CANCER ARE GETTING ACCESS TO QUALITY CARE.

By Michael G. Sheffield

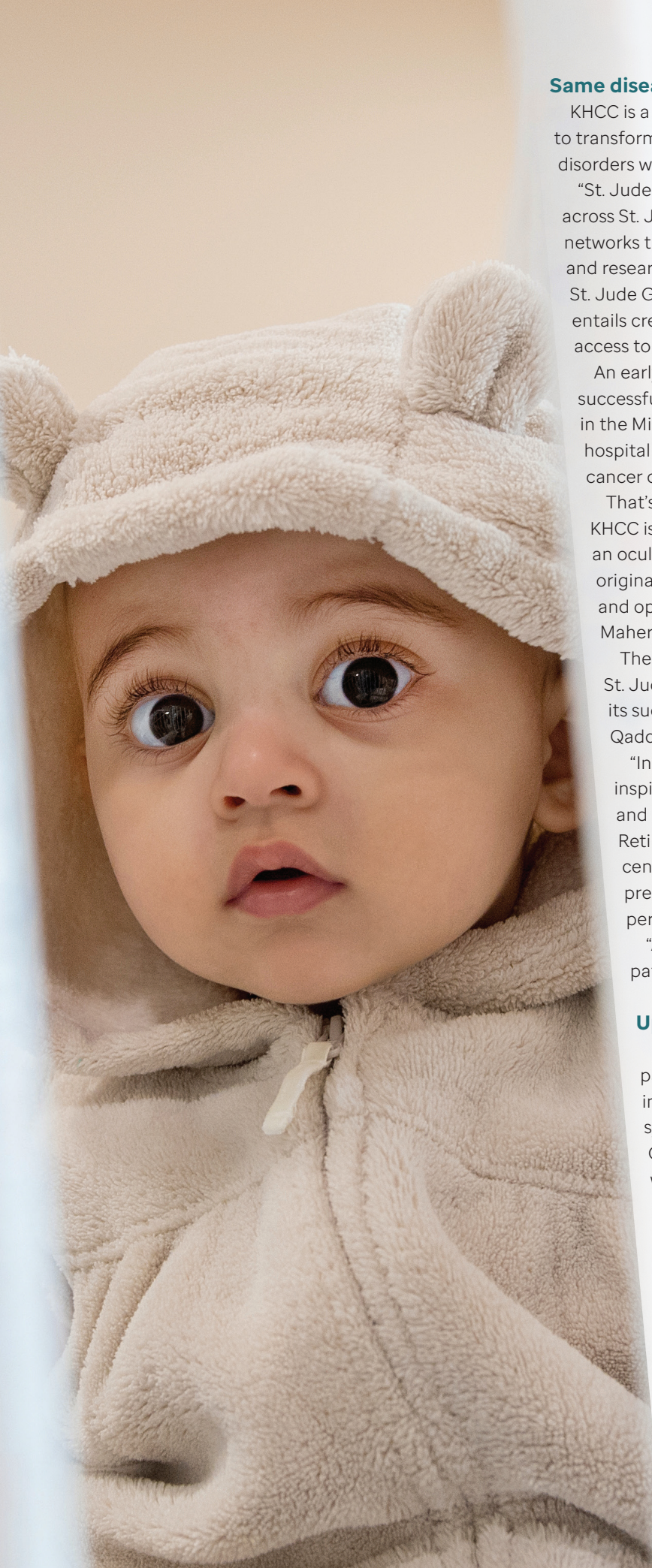
AS A CHILD IN SYRIA, Aber Al Rehman Attar battled an eye cancer called retinoblastoma. Without access to consistent medical care, he eventually lost his left eye, which surgeons removed. After the disease returned in his right eye, Attar was completely blind.

Years later, three of his five children were also found to have retinoblastoma.

However, their vision was saved, thanks to a partnership between St. Jude Children's Research Hospital and King Hussein Cancer Center (KHCC) in Amman, Jordan. Attar's 5-year old son, Suhaib, had begun treatment at 18 months old, after his mother noticed "something wrong with his eyes."

"We went to the doctor, found the cancer, and he was transferred to KHCC," Attar says. "He has completed treatment, and we're doing follow-up visits to make sure he's cancer free."

Although Suhaib's vision is impaired, his eyesight and life have been saved. His two other siblings were tested for retinoblastoma during infancy. Their treatment occurred early enough that they avoided vision issues. If the children have no recurrences before they turn 6, the chance of cancer returning is slight.



Same disease, different outlooks

KHCC is a regional partner for St. Jude Global, an initiative to transform the care of children with cancer and blood disorders worldwide.

“St. Jude Global allows us to harness talent and resources across St. Jude and around the world, developing strong regional networks that are focused around education, capacity-building and research,” explains Carlos Rodriguez-Galindo, MD, director of St. Jude Global and executive vice president. Capacity-building entails creating an infrastructure to ensure all children have access to quality care.

An early diagnosis of retinoblastoma in the U.S. often results in successful treatment, with little or no lasting effect. But patients in the Middle East may have to travel hundreds of miles to a hospital that’s unequipped to treat a serious illness, much less a cancer diagnosis.

That’s why the partnership between St. Jude Global and KHCC is so important, according to Yacoub Yousef, MD, an ocular oncologist and surgeon at KHCC. The program originated in 2003 with Ibrahim Qaddoumi, MD, of St. Jude, and ophthalmologists Ibrahim Al-Nawaiseh, MD, and Mustafa Maher, MD, in Jordan.

The partnership has grown under the leadership of St. Jude surgeon Matthew Wilson, MD. Yet Wilson attributes its success to “the two Ibrahims” (Al-Nawaiseh and Qaddoumi) and their passion for improving care for children.

“In short, the Jordanian retinoblastoma program is inspirational,” Wilson says. “If not for their partnership and passion, the program never would have started. Retinoblastoma care in Jordan never would have been centralized, and we would not have witnessed the precipitous drop in retinoblastoma mortality from 40 percent to 3 percent in two decades.

“As I reflect on my career in treating retinoblastoma patients, this may be the pinnacle,” he adds.

Up to date

Yousef estimates KHCC sees 14 or 15 retinoblastoma patients per week, coming from across the Arab world, including countries where previously functional health systems have collapsed, such as Syria and Iraq. St. Jude Global has initiated a program for displaced children with cancer in Jordan, a country with the second highest number of refugees per capita in the world.

“St. Jude Global assists health care professionals and patient advocates in these Middle Eastern countries in building sustainable networks that optimize equitable access to quality care for children with cancer,” says

Wide-eyed innocence

A tiny patient awaits treatment at King Hussein Cancer Center, one of six regional St. Jude partners located in the Middle East.



Focus on families

“KHCC is a place where hope is reborn for patients and their families,” says Rawad Rihani, MD, of King Hussein Cancer Center.

Sima Jeha, MD, director of the East and Mediterranean Region for St. Jude Global. “Childhood cancer is largely curable, but the treatment is complex. While a Syrian child with cancer could have received appropriate care before the crisis, the diagnosis of cancer is equivalent to a death sentence for a displaced child.”

Before 2003, patients with retinoblastoma had one choice for treatment: removal of the affected eye. If therapy doesn’t begin quickly, the tumor could eventually grow and spread outside the eye. Once that happens, the odds increase that cancer will spread to the brain and other parts of the body. Before the KHCC program began, the 40 percent mortality rate existed because patients would wait until the tumor had spread beyond the eye before seeking treatment. That’s not the case anymore. Yousef is still working to educate patients and their families about the disease.

His message is simple: With early diagnosis, the cure rate is extremely high.

“You can describe treatment in three words now: up to date,” Yousef says. “I’m looking at patients who have the disease in both eyes, but it isn’t due to genetics; it’s due to receiving a later diagnosis. Most patients and families don’t know eye cancer can happen.”

“As I reflect on my career in treating retinoblastoma patients, this may be the pinnacle.”

– Matthew Wilson, MD

St. Jude care in Jordan

In addition to using social media to promote early diagnosis and treatment, a robust fellowship program allows Yousef to train doctors from other Middle Eastern countries in the care and treatment of retinoblastoma. The therapy parallels that used at St. Jude. The retinoblastoma service was built upon the principle of twinning. This process paired St. Jude experts with local health providers and

community leaders in other countries, sharing expertise and promoting self-sufficiency.

After completing their fellowships, physicians return to their home countries and teach others what they have learned. KHCC is one of six regional partners that St. Jude



Building networks, saving lives

“Childhood cancer is largely curable, but the treatment is complex,” says Sima Jeha, MD (right), director of the East and Mediterranean Region for St. Jude Global.

relies on to build capacity in the Middle East.

Rawad Rihani, MD, a pediatric oncologist at KHCC, says treating children according to published St. Jude clinical trials has provided some of the best outcomes for patients. As a result, KHCC has become a regional cancer center of excellence for childhood cancer treatment. By widely replicating this program, the outlook should improve for children throughout the region.

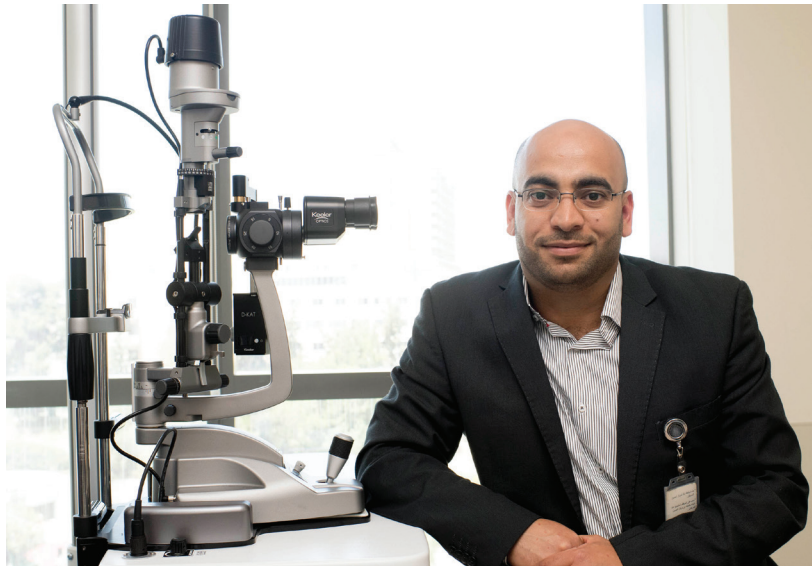
“KHCC is a place where hope is reborn for patients and their families,” Rihani says. “St. Jude was a partner from

the beginning, coming to visit the facility and working with our doctors.”

Looking toward the future

To determine the impact of St. Jude Global, look no further than Attar’s family. Lack of treatment options years ago resulted in his complete loss of sight. But early detection and treatment means his children have not only retained their vision, but have better chances for living normal lives.

“We can understand the disease and create better treatments for the future,” Yousef says. “You start in your house and you improve your home. Then you can help your neighbors, your city and country.” ■



Research, treatment and education

“You can describe treatment in three words, now: up to date,” says Yacoub Yousef, MD, of King Hussein Cancer Center.

St. Jude Global extends the hospital’s reach

More than 80 percent of children with cancer live in low- and middle-income countries. Most of those children will die from their disease.

Earlier this year, St. Jude launched St. Jude Global, a bold initiative to improve access to high-quality care for children worldwide. Within the next decade, St. Jude Global aims to increase the number of children whose care St. Jude influences from 3 percent to 30 percent. Ultimately, the goal is for all children with cancer to have access to quality care – no matter where they live.

The plan calls for action on three fronts:

Education: St. Jude Global will establish a comprehensive training program both in Memphis and at regional sites.

Program building and patient-centered care: St. Jude Global will develop regional partnerships with specific clinical care initiatives to advance care.

Research: St. Jude Global will facilitate research on a worldwide scale – enabling collaborative sites to perform high-quality and successful research projects.

St. Jude was recently named the World Health Organization’s first Collaborating Centre for Childhood Cancer. This designation represents a new push by both organizations to expand efforts that will advance pediatric cancer survival rates worldwide.



To Jordan and beyond

St. Jude Global aims to improve access to high-quality care for children worldwide.

Brain



Research that pairs brain stimulation and training shows promise in cancer survivors.

By Maureen Salamon



Helping tomorrow's survivors

St. Jude cancer survivor Mike Johnson volunteered to help researchers test a new tactic to boost working memory and executive function — the ability to retain information and solve problems.

Navigating a 30-year career as an insurance salesman requires Mike Johnson to constantly track his activities on a series of to-do lists. Now 66, Johnson could blame his need for memory-boosters on his age, but he doesn't do that. "I've been making lists for a long time," he says. "To me, it's just a way of life."

Johnson's cognitive challenges may also stem from his cancer battle as a teen in 1968. As one of the first 1,000 patients treated at St. Jude Children's Research Hospital, he was cured of acute lymphoblastic leukemia (ALL). But he and other survivors often face problems with working memory and executive function — the ability to retain information and solve problems — due to the disease and its treatment.

In an effort to address this issue, Johnson is helping St. Jude researchers by participating in a study. He is one of the first adult leukemia survivors to help test a combination of low-voltage brain stimulation with "brain training" games to boost thinking skills. Although the therapy has been used in other types of patients, this study is the first of its kind in childhood cancer survivors.

Early results are extremely promising, increasing brain function and potentially smoothing the futures of survivors and families, says Kevin Krull, PhD, of the St. Jude departments of Psychology and Epidemiology and Cancer Control.

"With successful treatment, typical childhood leukemia survivors are going to outlive their parents," Krull says. "If survivors have significant executive-function deficits and can't live independently, what happens when their parents are 70 or 80 and are more dependent on them? How does that affect the family dynamics?"

"We know those with higher executive function do better in getting higher-paying jobs and pursuing higher education, so they can attain a higher level of social independence and success," he adds.

Brain power

Many childhood cancer survivors report issues with memory and problem-solving. Kevin Krull, PhD, is working to remedy those challenges. In adult survivors of childhood leukemia, Krull and his colleagues have paired brain-training games with a process that delivers a low dose of electrical current to the brain.



Krull and his colleagues paired a technique called transcranial direct-current stimulation – delivering a low dose of electrical current to the brain – with brain-training games in adult survivors of childhood leukemia. All participants had shown problems with executive function, a skill that usually develops during childhood and adolescence.

“After just 10 sessions...participants said they could concentrate better, they got less distracted and could hold information in their heads better.”

–Kevin Krull, PhD

Scientists placed electrodes on the forehead to stimulate the brain’s frontal lobes, which are responsible for executive function. Survivors continued the 15-minute process at home for 10 sessions. Participants followed each session with 20 minutes of cognitive-training games.

“The notion was if they could do the cognitive training during the two-hour window of increased brain sensitivity after stimulation, they would learn the skill faster and it would help engage them more in the task,” Krull explains.

Johnson usually enjoyed the games, which had him virtually whizzing around racetracks, tracking the direction of flying birds or matching numbers on objects at the top and bottom of the screen. The games are meant to test skills such as working memory, processing speed and pattern recognition.

“Some were more intense or complex than others, and on some I was just trying to do the best I could,” he says. “I even continue to do some of them today. It was fun.”

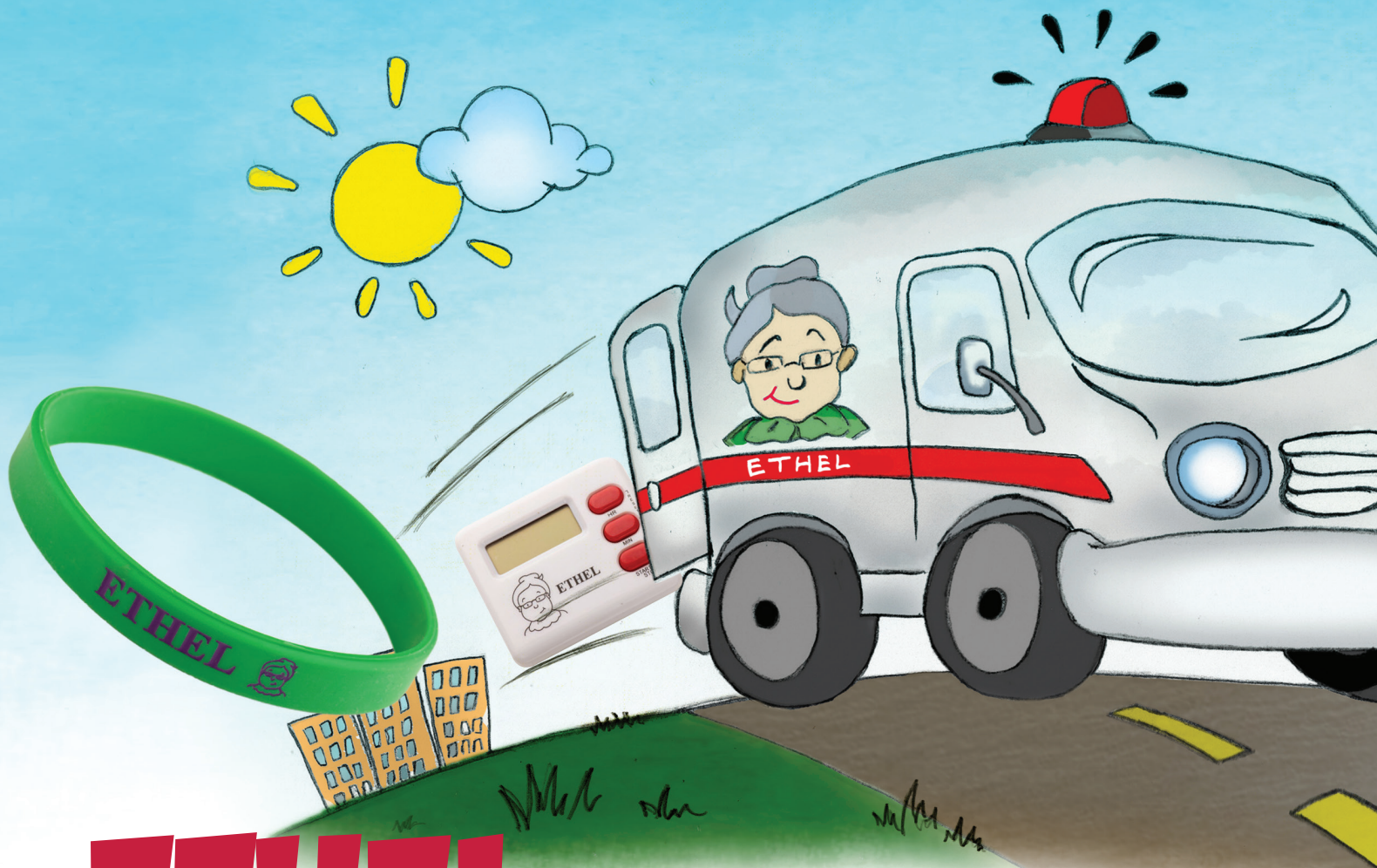


After the brain-training regimen, Johnson and fellow survivors completed the same cognitive tests they’d taken before the study. Results proved encouraging.

“After just 10 sessions, they improved both in their self-report of cognitive function as well as in our tests,” Krull says. “Participants said they could concentrate better, they got less distracted and could hold information in their heads better. I’m quite excited about it.”

The study’s findings will propel a much larger effort to determine whether this program can produce lasting improvements.

“Can we maintain this effect and magnify the effect over time?” Krull asks. “If so, we hope to begin treating children who are younger, who just begin to show problems, to hopefully prevent a lot of hardship down the road.” ■



ETHEL to the Rescue

How **ETHEL** helped researchers unmask an ineffective treatment used worldwide.

By Chris Pennington

Hannah Duckworth hangs onto things that matter.

Part of her collection from the time she received treatment at St. Jude Children's Research Hospital is a little green bracelet, emblazoned with a line drawing of a woman named Ethel. The bracelet is cheap, but its significance is invaluable. It's the reminder of a clinical trial in which Duckworth participated.

"My memory of that time is patchy," Duckworth says of her treatment for acute myeloid leukemia seven years ago. "But I do know that any time someone offered a study, I wanted to help out where I could."

Duckworth is one of about 90 patients who participated in the first study of its kind, to find out whether a treatment commonly used around the world is actually effective.

Lifeline for patients

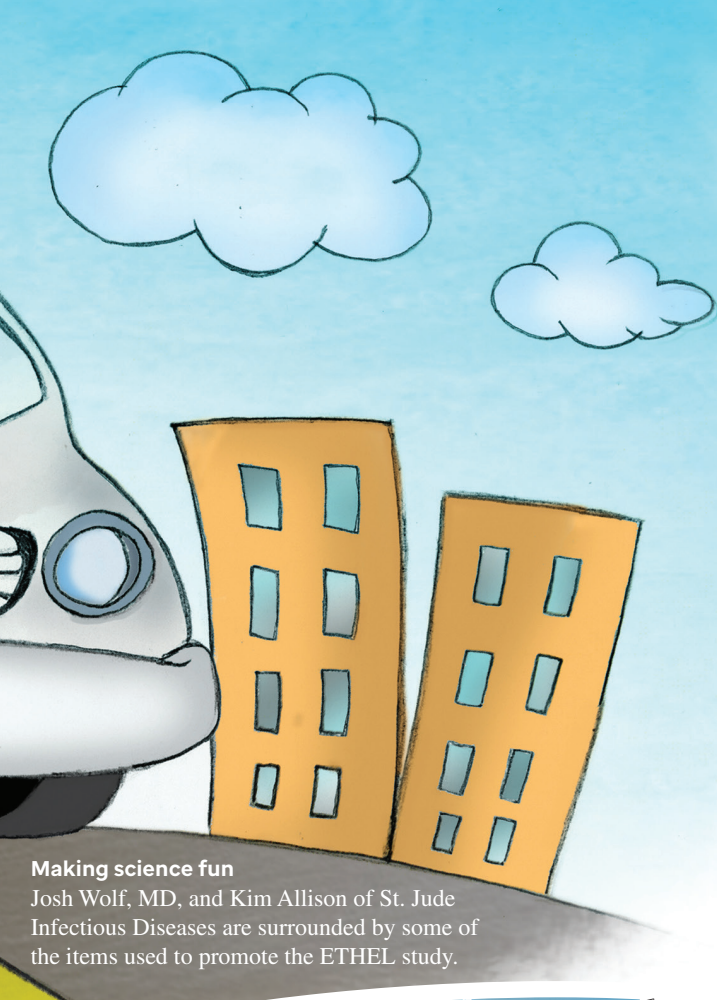
Most children undergoing cancer treatment have central venous catheters, known as central lines, surgically inserted into their chests. Connected to the bloodstream, the central line allows clinicians to draw blood and deliver fluids and medicines without repeated needle sticks.

Although they're lifesaving, central lines can also cause problems. About a fourth of cancer patients develop central-line infections during therapy; this is caused by bacteria or other germs building up in the line and spreading to the bloodstream. These infections require hospitalization and delay cancer treatments. In some cases, the infections require surgical removal of the central line or can even be life threatening.

"I got an infection in my lung and line," Duckworth says. "It was a wild trip and an extremely brutal treatment."

A difficult sell

Many hospitals use ethanol, a type of alcohol, to prevent or treat central-line infections. In ethanol-lock therapy, an ethanol solution is placed into the central line to kill bacteria.



Making science fun

Josh Wolf, MD, and Kim Allison of St. Jude Infectious Diseases are surrounded by some of the items used to promote the ETHEL study.



In the lab, ethanol effectively kills bacteria in central lines. But is the treatment effective in humans?

That's what Josh Wolf, MD, of the St. Jude Infectious Diseases Department, wanted to know. He and his colleagues teamed up with clinicians in Australia to find the answer.

As part of a clinical trial, patients were randomly assigned to a six-month schedule of central line treatment. Half were treated with ethanol therapy and half with the normal heparin-saline solution.

The study required an enormous amount of coordination and work, involving the assistance of patients and families, as

well as physicians, nurses, clinical teams, pharmacy staff and others.

It was a difficult sell.

"We needed to recruit patients for the study and then educate them about study guidelines," Wolf says. "We asked hundreds of staff members to do something for us in addition to their day-to-day duties. On top of this, we asked patients to participate in a therapy where they didn't know what they would receive."

Friendly reminder

Providing something small, consistent and recognizable could tie all the departments and patients together – alerting everyone and reminding them about the study. The answer was ETHEL, short for ethanol-lock therapy.

"ETHEL became the image for communicating through this study," Wolf says. "ETHEL's image greeted every message we sent to those involved."

Wolf used whatever he could to keep ETHEL front and center for patients and staff – pizza and donuts for nursing staff, milestone messages marking increased enrollment, bracelets that identified patients as part of the study.

It worked. Participation was high, with both staff and patients engaged in the process.

"My feeling was that this portion of my time could really change the future," Duckworth says. "Processes were being questioned and things were getting figured out. I'm glad I participated, because it helps patients now."

Surprising outcome

The ETHEL study had results that were definitive, but unexpected.

About half of the patients in both groups developed new or recurring infections, and nearly six of every 10 patients in the ethanol group needed blood thinners to open blocked central lines. Only a third of the standard treatment group required similar thinners.

In short, the study found that ethanol-lock therapy doesn't work to treat and prevent infections, but that it does increase the risk of line blockages.

"That's important to know," Wolf says. "We need to make sure we never use treatments that cause more harm than good."

Today, Duckworth is a college student who has aspirations of helping the hearing impaired. She also derives satisfaction from knowing future patients can benefit from her experience at St. Jude.

"While you're there, you help out where you can," Duckworth says. "Participating in the study was five minutes of my time, and it helped others down the line." ■

SEE, HEAR and BREATHE the DIFFERENCE

Otolaryngology services at St. Jude grow in scope, complexity and reputation.

By Maureen Salamon

During a two-week span last winter, a pea-sized lump on Belle Grochowski's cheek grew so large that the toddler couldn't breathe out of her right nostril or fully see from her right eye. Misdiagnosed at her local hospital, Belle and her alarmed parents obtained a referral to St. Jude Children's Research Hospital. There, they hoped to find not only clarity, but also hope and quick action.

They found all three at St. Jude, where doctors correctly identified the mass as a desmoid tumor, a noncancerous but aggressive growth that can develop randomly almost anywhere on the body. Because of the rare tumor's location on Belle's face, however, removing it was paramount – and precarious.

The complex surgery had the potential for leaving the chatty, outgoing toddler with extensive scarring, if not worse. But the

hospital's surgeons aimed to avoid that scenario. A trio of specialists collaborated in the operating room to remove most of Belle's tumor through her nostril and sinus cavity without making any skin incisions. To the naked eye, she looks virtually untouched.

"I was expecting the worst," recalls her mom. "But you can't tell anything has happened to Belle. They really preserved her quality of life."

Experts in demand

Otolaryngology focuses on diseases and disorders of the ear, nose and throat (ENT), as well as related structures of the head and neck. At St. Jude, more than 100 children undergo ENT procedures each year, with about 700 receiving ongoing treatment at outpatient clinic visits.

"The number of children in our service is higher than people might expect," says Tony Sheyn, MD, who led Belle's surgical team. "I was quite surprised at the high



Tony Sheyn, MD

SETH DIXON

volume when I came here, but there's definitely an opportunity to continue building on what we already have."

Sheyn's arrival in 2015 coincided with a growth spurt in otolaryngology services at St. Jude – not just in size, but also in scope and complexity. The group's eight physicians, nurse practitioners and other clinicians handle a wide array of conditions and treatments ranging from sleep apnea to thyroid and salivary gland tumors to cochlear implants.

The team approach

Working with other St. Jude departments, ENT doctors have also



PETER GARTIA

begun developing a voice rehabilitation program in addition to established programs in swallowing and hearing rehabilitation. Otolaryngology doctors at St. Jude partner with head and neck cancer surgeons as well as with a skull-base surgeon who performs surgery on the bony surface beneath the brain. This specialized group can tackle increasingly complex operations most other centers can't offer.

"The team mentality at St. Jude is fantastic, and anybody who comes here feels that," Sheyn says. "I think it's consistent with what St. Jude is known for, because our mission includes treating children with rare tumors and cancers."

"It's important to have not only otolaryngology, but all of the surgical subspecialties well covered for St. Jude patients," adds Andrew Davidoff, MD, St. Jude Surgery chair. "ENT is an important part of that."

The secret's out

As the ENT program has grown, so has its reputation. On Sheyn's watch, St. Jude has developed one of the busiest thyroid programs in the country, forming a multidisciplinary thyroid team that includes specialists from oncology, endocrinology, nuclear medicine, pathology, diagnostic imaging, psychology, genetics, general pediatric surgery and ENT.

Thyroid cancer among children remains extremely uncommon, but St. Jude receives referrals from across the region because of the group's deep expertise. Sheyn has consulted on more than 100 such patients in the last three years.

"Word has gotten around that we're accepting these patients for treatment here and have an expert with an interest in thyroid cancer," Davidoff says.

A referral to St. Jude can spell the difference between a good and great

Dramatic transformation

St. Jude surgeons removed a fast-growing tumor from the face of Belle Grochowski (shown at right before surgery). Today, Belle is a bright and playful 2-year-old (above) with no facial scars.



result for a child with an unusual ENT condition. Belle's mom says the 2-year-old is back to greeting everyone she meets in the grocery store, with no indication of her recent medical ordeal.

"She had a very good cosmetic result, and that demonstrates the team approach to how we treat kids here," Sheyn says. "It's a great outcome for a small child who won't have any external marks as a result of her surgery." ■

STRENGTH in NUMBERS

The syndrome is rare, the implications are daunting. But one life conference at St. Jude empowers families who have Li-Fraumeni syndrome.

By Elizabeth Jane Walker



NEISHA T. FORD PHOTOGRAPHY

Extending the support system

“At the conference, I was able to meet people who’ve had bouts with cancer over and over, and they’ve beat it,” says Neisha Ford, pictured with sons Leo (at left) and Cyrus. “I met others who’ve gotten cancer once and never had it again. And some people had never gotten cancer at all. It was inspiring and comforting to be around all of those people.”

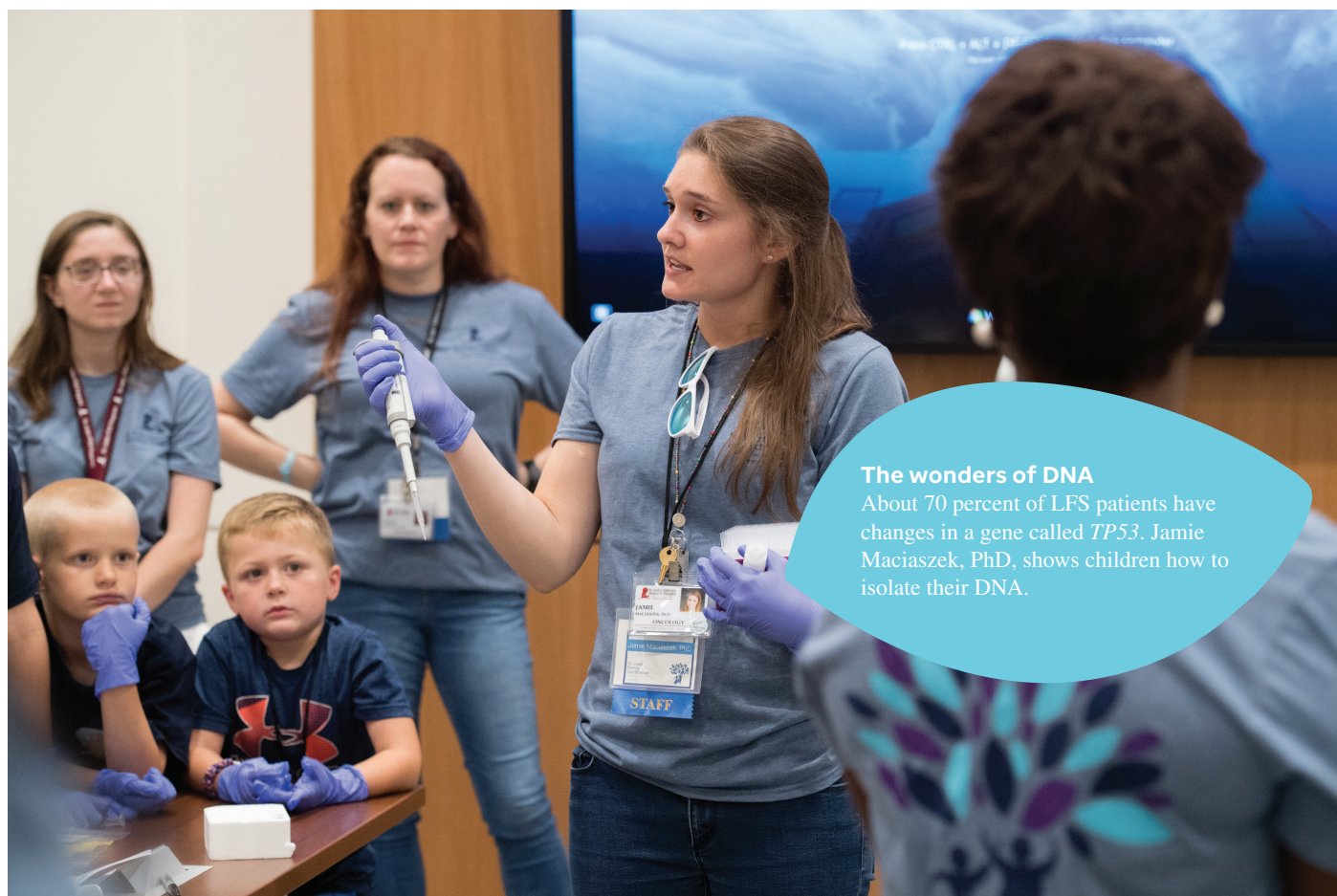
With his luxuriant hair, engaging smile and lively intelligence, Cyrus Ford draws admirers like a magnet attracts iron. As Neisha Ford listens to her son’s constant chatter and watches him sprint after his big brother, she contemplates the challenges Cyrus has overcome in his three short years of life.

When he was 5 months old, Cyrus was diagnosed with a rare form of pediatric cancer. Neisha and Shawn Ford learned their son also had a rare, inherited disorder called Li-Fraumeni syndrome (LFS). People with LFS have a high likelihood of developing one or more cancers in the brain, breasts, bones, blood, muscles or, in the case of Cyrus, the adrenal gland.



According to Kim Nichols, MD, director of the Cancer Predisposition Division at St. Jude Children's Research Hospital, half of all individuals with LFS develop some type of cancer by age 30. By age 60, that risk skyrockets to 80 to 90 percent.

Nichols says people with the disorder should learn as much as possible about the condition so they can monitor for cancer and minimize their risk of developing it. She and her colleagues recently hosted the St. Jude Family Conference on Li-Fraumeni Syndrome. It's the first in a series of events designed for families with hereditary predispositions to cancer.



The wonders of DNA

About 70 percent of LFS patients have changes in a gene called *TP53*. Jamie Maciaszek, PhD, shows children how to isolate their DNA.

Friendship + knowledge = hope

When the Fords arrived at St. Jude for the conference, they were eager to meet other people with LFS. Ninety-six individuals from 25 families across the nation attended the event. Participants bonded through activities that included a Memphis Redbirds baseball game as well as lectures and interactions with world-renowned LFS researchers and clinicians. Because early detection can save lives, attendees learned about cancer screenings and gleaned tips for leading healthful lifestyles.

"Until this conference, I'd only met two other kids who had Li-Fraumeni syndrome," Neisha says. "I have an amazing support system, but not knowing anyone else with Li-Fraumeni was really isolating. At the conference, I was able to meet people who've had bouts with cancer over and over, and they've beat it. I met others who've gotten cancer once and never had it again. And some people had never gotten cancer at all.

"It was inspiring and comforting to be around all of those people."

Inspiration and motivation

About 70 percent of LFS patients have changes in a gene called *TP53*. Emilia Pinto, PhD, of St. Jude Pathology has spent years studying adrenal tumors and this gene in the lab. She performed the genetic tests that uncovered Cyrus' *TP53*

mutation, but had never met the Fords until the St. Jude Family Conference.

"It was amazing to see the families together," says Pinto, who shared her expertise at the event. "They're all dealing with the same problems and are so supportive of each other."

Pinto says meeting the participants adds a new urgency to her research.

"I have a huge passion for my work," she says, "But after meeting these families, I know we need to do more. It's important to do more."

Looking ahead

Many of the conference's participants report the experience was life changing.

Neisha says she gleaned valuable tips from a session in which Niki Jurbergs, PhD, of the St. Jude Psychology Clinic offered tips for talking about LFS with children and other family members.

"It's made a big difference in how we communicate," Neisha says. "We were walking out the door the other day, and my 4-year-old said, 'Hey, Cyrus, I'm glad you don't have cancer anymore, because you're awesome and you fight the best.' And Cyrus said, 'Yeah, I kick cancer's butt.' That conversation is possible because of what we learned at the conference."

Patty and Bob Leffler traveled from Wisconsin with their three children to attend the conference.

"We met people who are dealing with LFS, and dealing with it well," Patty says. "Some people had been diagnosed four or five times with cancer. That's hard to hear, but they aren't sitting around feeling sorry for themselves. They're getting married and having kids and enjoying their lives and

trying to learn more about Li-Fraumeni so that they can continue to live great lives.

"As another St. Jude mom once told me, 'Nobody is given a good life or a bad life. We are simply given a life, and it is up to us to make it a good one.'" ■

Learn more about cancer predisposition: stjude.org/cancer-predisposition

"I have a huge passion for my work. But after meeting these families, I know we need to do more. It's important to do more."

- Emilia Pinto, PhD



Un-bearably cute

The sale of huggable animals has helped Signet Jewelers raise \$66 million for St. Jude.



Holidays with Heart

St. Jude celebrates 15 years of *Thanks and Giving*.

By Grace Korzekwa

With the creation of their first plush, named Cubby, longtime partner Signet Jewelers gave customers a reason to remember St. Jude Children's Research Hospital during the holiday season.

Through 15 years of the St. Jude *Thanks and Giving* campaign, Cubby's family of adorable plush has grown. There are Aiden and Kip and Travis and many more – two new additions each year, each tested for huggability. These bears and puppies have helped Signet celebrate special moments with its customers and raise \$66 million for St. Jude since the partnership began.

"Signet is honored to support the work of St. Jude through its *Thanks and Giving* campaign," said Gina Drosos, CEO of Signet Jewelers, parent company of Kay Jewelers®, Jared The Galleria of Jewelry®, Zales® and Piercing Pagoda®. "Since joining the *Thanks and Giving* campaign when it began 15 years ago, Signet team members across the country have felt a tremendous sense of pride each year toward supporting such a worthy cause around the holidays."

Just as the winter holidays wouldn't be complete without the latest holiday plush, St. Jude *Thanks and Giving* wouldn't work without its corporate partners.

"When Tony, Terre and I came up with the idea for *Thanks and Giving*, we wanted to provide America's most beloved companies and their customers with an easy way to support the children of St. Jude," says Marlo Thomas, the hospital's national outreach director. "Year after year, our partners continue to amaze us with their tireless commitment to the lifesaving mission of St. Jude. As a result of their support, we are able to stay dedicated to our father's founding promise that 'no child should die in the dawn of life.' There's more work to be done, and we won't stop until no child dies of cancer."



Raising dough

St. Jude patient Isabelle Richard tosses pizza dough with guidance from Domino's employees.

Domino's chooses St. Jude again

After their first campaign with St. Jude in 2004, Domino's franchisees voted to make the hospital their national charity of choice. Since then, Domino's has integrated St. Jude into every step of its growth as a business, including the design of its pizza boxes. Tim McIntyre, Domino's executive vice president of Communications, Legislative Affairs and Investor Relations, fostered the company's relationship with St. Jude from Day One. He even ran a half marathon for St. Jude.

"Thanks and Giving was an incredible, inspirational idea, and it captured our imaginations here at Domino's – even if we didn't quite know how to approach it at first," McIntyre says. "We went from raising just over \$350,000 the first year to \$9 million last year. It's rewarding for all of us to be part of something greater than ourselves."

From cash register to big screens

Holiday shoppers at Marshalls have supported the hospital for 15 years, one cash register add-on at a time. Through St. Jude *Thanks and Giving*, the retailer has raised more than \$11 million for the hospital since 2004.

Regal Entertainment Group, the second-largest theater chain in the country, has used its 7,000-plus screens to bring a special St. Jude message to moviegoers every holiday season for 15 years. And there's another special gift Regal gives the children of St. Jude: an exclusive movie screening. Recently, patients walked a red carpet at St. Jude to watch the premiere of *Paddington 2*, complete with popcorn and candy, ahead of the theatrical release.

Thanks for giving

Marlo Thomas, national outreach director, celebrates St. Jude *Thanks and Giving* with Nikalis Kelly (left) and Bridget Korn.



2 fun

Ben Krizhanovskiy (left) and his brother Joshua arrive for the premiere of *Paddington 2*.

More love for St. Jude

These committed companies are among more than 60 brands that are part of St. Jude *Thanks and Giving* campaign this year, including Best Buy, Kmart, HomeGoods, Ann Taylor, Williams Sonoma, Chili's, AutoZone, New York & Company and many more.

In addition to generous corporate partners, St. Jude *Thanks and Giving* features celebrities who donate their time and voices to share the hospital's mission. This year, watch for ads that include Jennifer Aniston, Luis Fonsi, Jon Hamm, Michael Strahan and Sofia Vergara, who join Marlo Thomas alongside St. Jude patients.■



TURN UP THE RADIOCHEMISTRY

Scientists in the St. Jude Molecular Imaging Core race against time to produce PET radiotracers to diagnose illness and observe many biochemical processes.

By Mike O'Kelly

From cyclotron to clinic

Eric Erdman undergoes a PET scan, which uses radioactive materials produced in the St. Jude cyclotron.

It's a car-sized, state-of-the-art machine with a name that sounds out of this world. Although the cyclotron at St. Jude Children's Research Hospital doesn't blast off to distant planets, it spins possibilities to rival any science fiction tale.

Resting under 7 feet of concrete in an underground bunker, the cyclotron serves as the foundation for work done in the hospital's Molecular Imaging Core.

The cyclotron creates radioactive chemicals that are used to make drugs, known as radiotracers, for a procedure known as positron emission tomography, or PET imaging.

Amy Vavere, PhD, directs the Molecular Imaging Core, which launched as a resource within the Department of Diagnostic Imaging earlier this year.

The Molecular Imaging Core enables St. Jude researchers to use nuclear medicine and nuclear chemistry in their studies. Although the most common PET radiotracer, FDG, is available from a local vendor, other radiotracers are made onsite at the Molecular Imaging Core, an advantage for researchers and clinicians.

"We support research across the institution, and our work crosses over into the clinic," says Vavere, who joined St. Jude in 2007, shortly after the cyclotron arrived on the hospital's campus. "We can measure all kinds of biological processes using radiotracers."

PET project

Radiotracers can diagnose illness, measure blood flow, monitor tumor growth and track response to therapy. For instance, a patient with a brain tumor may undergo frequent PET scans to track treatment response. As part of the procedure, the child lies flat on the scanner while an imaging

technologist injects the radiotracer.

Barry Shulkin, MD, chief of Nuclear Medicine, monitors the radiotracer as it travels through a child's body.

"A PET scan shows what is going on inside the body in real time," he says. "Not only does it capture a snapshot of what is happening, but it also provides details into the functions of what you're seeing. It is functional imaging that you don't get from an MRI or CT scan."

PET imaging is an important asset at St. Jude. When a combination of MRI or CT scans yields conflicting readings, PET imaging often provides the information clinicians need to make treatment decisions. Physicians can use PET to decide what levels of radiation or chemotherapy are needed as well as examine radiation's effect on a tumor.

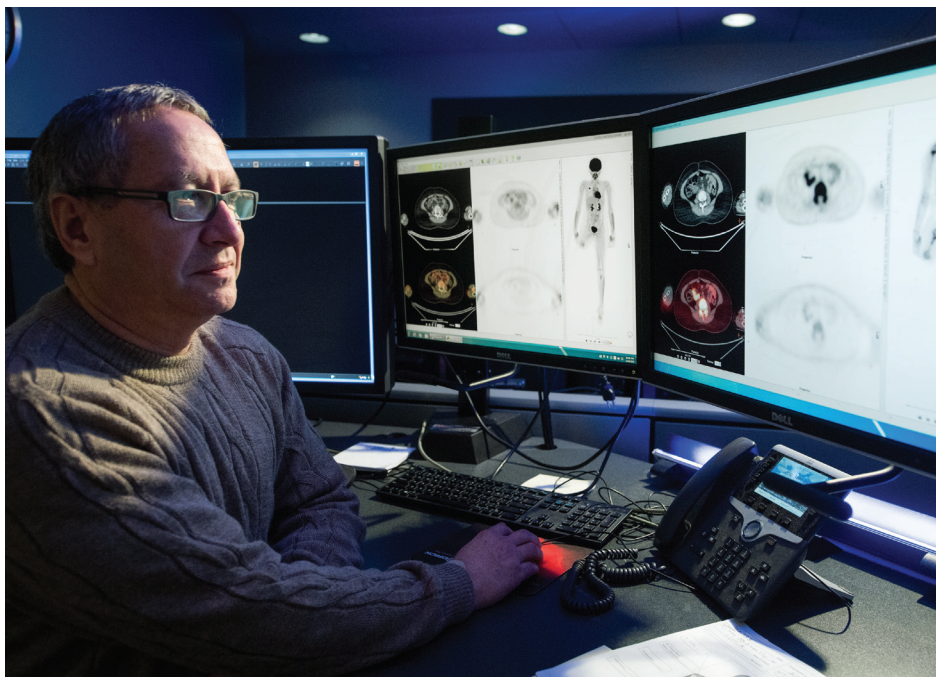
Dancing to the radio

The challenge with PET scans is radiotracers decay quickly – some last up to a few hours while others dwindle much

more rapidly. This decay rate, known as a half-life, is crucial to planning. It's why Molecular Imaging Core staff coordinate their efforts like a well-choreographed dance when a patient requires a specialty PET scan.

Half-life refers to the amount of time a radioactive element takes to decay in half. The most used tracer produced by the Molecular Imaging Core has a half-life of 20 minutes. Every 20 minutes, half of the usable radioactivity has decayed, meaning the team must act quickly and make enough radioactivity to prepare the radiotracer to leave time for quality testing before sending it for injection. Vavere compares this process to a melting block of ice.

"Imagine that you have a block of ice the size of a loaf of bread," she says. "The half-life tells you that in that amount of time, half of the ice, or loaf, will be gone. What remains is only half as much, and it's still potent and efficient at cooling. Even though only half of the radiotracer



The secrets within

Barry Shulkin, MD, chief of Nuclear Medicine, monitors a radioactive chemical, known as a radiotracer, as it travels through a patient's body. Radiotracers can diagnose illness, measure blood flow, monitor tumor growth and track response to therapy. These radiotracers are created at St. Jude in the Molecular Imaging Core.

is remaining, it is still just as effective at doing its job, emitting positrons that we can detect.”

Racing the clock

If a patient is scheduled for a PET scan at 10 a.m., chemists begin setup at 7:30 a.m., calibrating, sterilizing and cleaning all equipment. Access to the Molecular Imaging Core is limited; everyone who enters must wear lab coats, safety glasses and radioactivity detectors.

“Everything is coordinated to the minute. We put in a buffer of a few minutes, but if there is too much buffer time, then we have to start with a lot of extra radioactivity to end up with the amount the doctors need for a good PET scan,” Vavere says.

The beat goes on

The Molecular Imaging Core enables investigators to use radiotracers to see how drugs are delivered in preclinical

models and to develop new radiotracers.

“We’re here to help St. Jude clinicians and researchers use this process to image various biochemical processes and target expression using PET imaging, which offers physicians a higher level of functionality when making treatment decisions,” Vavere says.

“We want to foster stronger and more collaborative work so our patients have access to the best this technology has to offer.” ■



BIRTH OF A RADIOTRACER

Many meticulous steps must occur for a radiotracer to be used in a PET/CT scan:

- **After cleaning is complete** and components have been set up, the engineer fires up the cyclotron to begin its work.
- **A nonradioactive material** such as pressurized gas is placed into one of eight small chambers around the cyclotron’s perimeter.
- **Hydrogen gas is sent through a high-voltage electrical current** to create a high-energy beam of ionized gas.
- **The hydrogen ions are accelerated** by a strong electric field produced by an amplifier. Meanwhile, two large electromagnets steer the ions from the cyclotron’s center to the chambers on the outer edges.
- **The beam of protons is aimed at the gas inside the target**, converting it from one element to another. The material is radioactive, but not ready for use.
- **The material is pumped into one of eight lead-shielded hot cells**, where a chemist uses remote-controlled machines to convert the chemical into a drug.
- **The resulting radiotracer is sterilized** by filtering it into a glass vial.
- **The dose vial is transferred into a cabinet called an isolator**, where a robot fills syringes with a predetermined amount of the radiotracer specific to the patient.
- **A small amount of that radiotracer is tested** for quality and sterility in the lab’s Quality Control room.
- **A pharmacist verifies** the dose has passed required tests and approves its release.
- **The dose is placed into a lead container** and sent through a pneumatic tube to the Nuclear Medicine Clinic, where staff members conduct more safety checks.
- **The patient receives the drug.**



Thinking inside the box

Victor Amador Diaz and Amy Vavere, PhD, work on machinery inside one of eight lead-shielded hot cells, where chemicals from the cyclotron are converted into radioactive drugs.

news**highlights**

NCI awards ‘exceptional’ rating to St. Jude Comprehensive Cancer Center

For the second time in a row, the National Cancer Institute (NCI) has awarded St. Jude the highest possible rank of “exceptional” during renewal of the hospital’s Comprehensive Cancer Center grant.

St. Jude has the first and only NCI-designated Comprehensive Cancer Center devoted solely to children. The designation and accompanying grant funds must be renewed every five years; St. Jude first earned an “exceptional” score in 2013. St. Jude has had the designation as an NCI Cancer Center since 1977.

“Our center forms the heart of our vision for cancer research at St. Jude, encompassing efforts from our laboratories to clinical trials for our patients,” said Charles Roberts, MD, PhD, St. Jude executive vice president and Cancer Center director.



Rhabdomyosarcoma discoveries move from lab to clinic

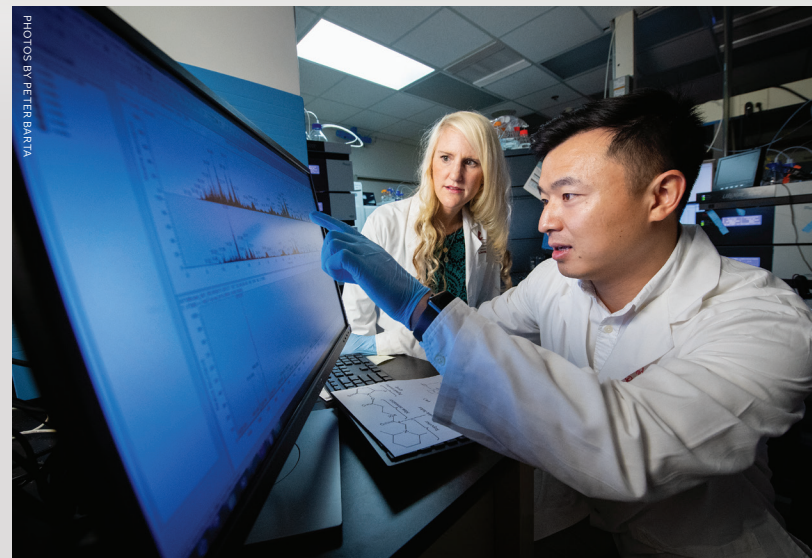
St. Jude scientists have completed the most complete analysis yet of the muscle and soft tissue tumor rhabdomyosarcoma. The researchers found weaknesses they can target. They also discovered a promising precision medicine for the disease.

Cure rates are 75 percent for patients whose rhabdomyosarcoma has not spread. But for children whose cancer returns or spreads, long-term survival is 30 percent or less.

Researchers learned that the two main varieties of rhabdomyosarcoma occur at different developmental times.

They also found several signaling pathways that are disrupted in tumor cells. A drug called AZD1775 inhibits an enzyme in one of these pathways. Scientists screened more than 1,700 drug-tumor combinations as part of the project. As a result, the Children’s Oncology Group expanded a clinical trial of AZD1775 to include children with high-risk rhabdomyosarcoma.

The research, part of the St. Jude–Washington University Pediatric Cancer Genome Project, appeared in the journal *Cancer Cell*.



Elizabeth Stewart, MD (left), and Hong Wang, PhD

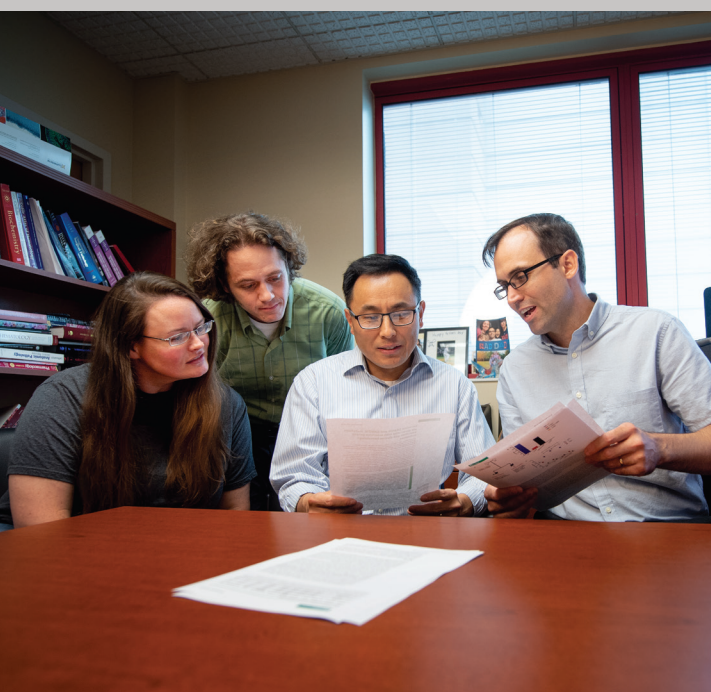
St. Jude partners to improve knowledge worldwide

St. Jude and the Institute for Health Metrics and Evaluation at the University of Washington are partnering to advance knowledge and understanding of childhood cancer around the world. The collaborators will collect and share data, leveraging their expertise in health metrics sciences to provide more accurate estimates of the global burden of cancer among children.

“This collaboration will yield vital information to inform health service delivery and ensure that every child has access to quality cancer care regardless of where he or she lives,” said Carlos Rodriguez-Galindo, MD, St. Jude executive vice president and chair of Global Pediatric Medicine.

Carlos Rodriguez-Galindo, MD

Discovery may help some children avoid bone marrow transplantation



From left: Tamara Lamprecht, Jason Schwartz, MD, Jing Ma, PhD, and Jeffery Klco, MD, PhD

Researchers have solved a decades-old mystery about which mutations cause a rare, inherited bone marrow disorder. The answer may allow some children to avoid bone marrow transplantation.

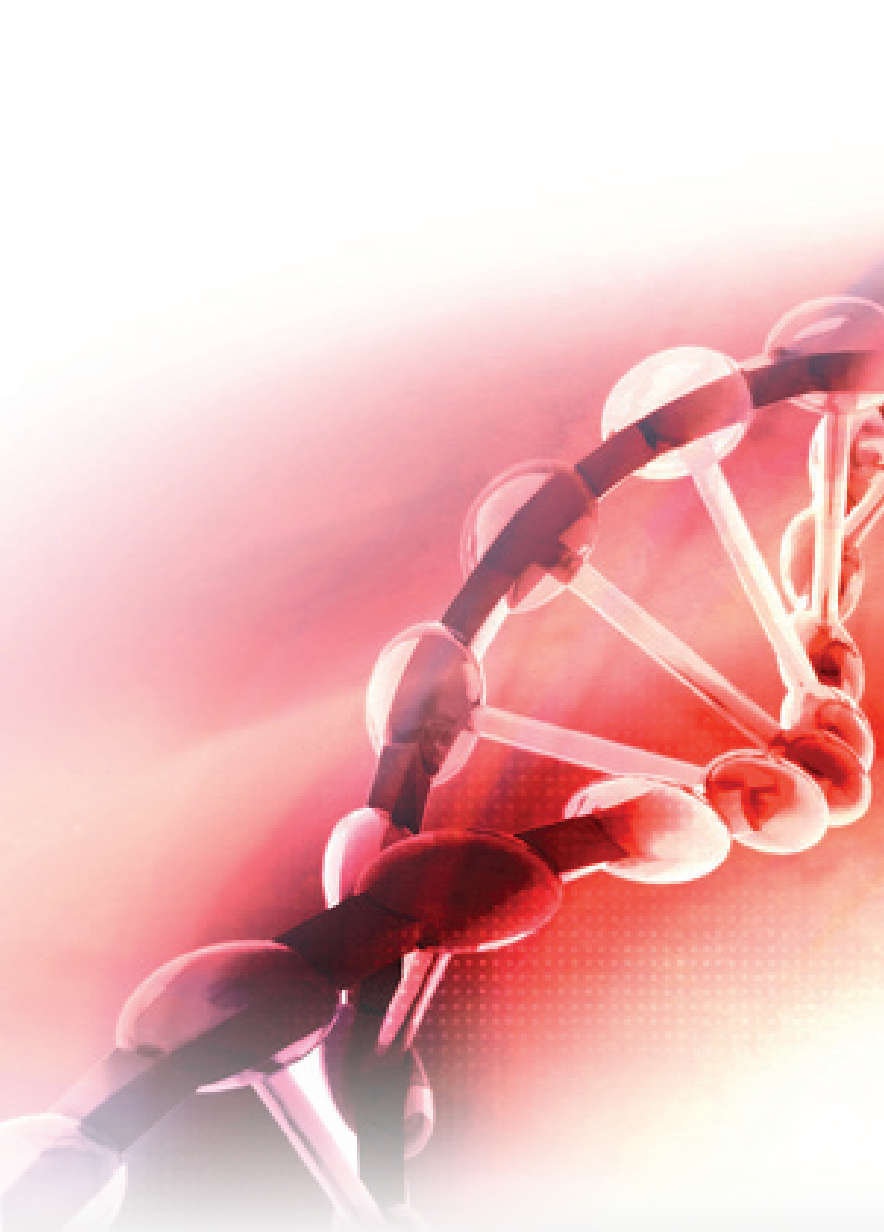
Scientists studied blood samples from 16 siblings in five families affected by myelodysplasia and leukemia syndrome with monosomy 7, which is also called familial monosomy 7 syndrome. All 16 siblings were found to have germline mutations in the genes *SAMD9* or *SAMD9L*.

Surprisingly, 11 of the children regained normal bone marrow function.

“Now that we know this disease can resolve without treatment in some patients, we need to focus on developing screening and treatment guidelines,” said Jeffery Klco, MD, PhD, of St. Jude Pathology. “We want to reserve bone marrow transplantation for those who truly need the procedure. These findings will help to point the way.”

The study identified *SAMD9* and *SAMD9L* as cancer predisposition genes that should be included in genetic counseling and screening for at-risk patients and families.

A report on this study appeared in the journal *JCI Insight*.



Genetic counseling suggested for all childhood cancer survivors

A St. Jude study suggests all childhood cancer survivors should consider genetic counseling to find out if they are likely to benefit from genetic testing.

St. Jude offers this service to every new patient with cancer. The counseling is also offered to St. Jude cancer survivors.

The study involved whole genome sequencing of 3,006 St. Jude childhood cancer survivors. Almost 6 percent of those survivors had germline (likely inherited) mutations in one of 60 genes that can lead to cancer. The number of mutations in this group was about 10-fold greater than among people with no childhood cancer history.

The study marks the first time whole genome sequencing has been used in a large group of childhood cancer survivors to study how genes affect lifetime cancer risk.

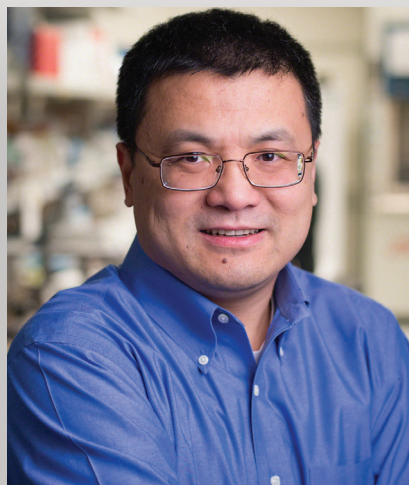
A report on this study appeared in the *Journal of Clinical Oncology*.

Scientists identify a protein complex that shapes T cells' destiny

Like a mentor helping medical students choose between specialties, a protein complex helps shape the destiny of developing T cells, according to St. Jude scientists.

The scientists learned how a protein complex called mTORC1 influences whether cells become conventional or unconventional T cells. Disrupting mTORC1 led to changes that favored development of unconventional T cells.

The research comes amid excitement about harnessing the immune



Hongbo Chi, PhD

system to fight cancer, tame autoimmune diseases and combat infectious diseases.

"We know that conventional and unconventional T cells are fundamentally different," said Hongbo Chi, PhD, of St. Jude Immunology.

"They express different cell surface receptors. The cells have different functions. But until now the mechanism that helps decide their fates has remained largely unknown."

The research appeared in the journal *Science Immunology*.



Infinite Love and Gratitude

From holy ground to hallowed halls

After completing treatment at St. Jude, Somer Greene (far right) went on to earn a master's degree at the University of Cambridge in England.

After pain, loss and incredible challenges: A life of joy, service and possibilities.

By Somer Greene

It was the evening of March 1, 2011. The restaurant was nearly empty: a waiter hummed, candles glowed. In that quiet space, my mother told me my medical test results.

At age 17, I had hereditary diffuse gastric cancer.

My father had been battling this disease for over a year. Now, we learned I had inherited the *CDH1* gene mutation, and it was already producing cancer cells.

A few weeks later, surgeons removed my stomach and part of my esophagus. This surgery removed cancer cells and prevented them from returning. However, as I was recovering, my dad's health was declining. Unfortunately, he passed away a couple of weeks after I left the hospital.

At St. Jude Children's Research Hospital, my oncologist, Alberto Pappo, MD, navigated these scary experiences with me. My cancer was extremely rare. It's an adult cancer — kids aren't supposed to have it — and after my father's death, Dr. Pappo became a father figure to me. When my brother suddenly passed a year later, my medical team of doctors, nurses, social workers and dietitians showed extraordinary compassion as they helped me find strength in my mourning.

St. Jude was my only constant then, a sacred place where I felt safe in my smiles and optimism even as I embraced the

unknown. My medical team created a space for me to understand cancer as an illness, not a finale. I have infinite love and gratitude for them, and for those who support the hospital.

After cancer came college, and after college I earned my master's degree in sociology at the University of Cambridge in England, where I studied the sociology of fundraising media. I'm now a fundraising officer there, thanks to the tremendous love and support of St. Jude.

A current of strength runs through St. Jude. I'll never understand how something as scary as cancer became the stage for such remarkable unity. My gratitude only continues with each yearly check-up — gratitude for the holy ground of St. Jude, and to its beautiful people. ■

St. Jude was my only constant then, a sacred place where I felt safe in my smiles and optimism even as I embraced the unknown. My medical team created a space for me to understand cancer as an illness, not a finale."



Give to help him live.

St. Jude patient
Woods, age 4
brain cancer

Give thanks for the healthy kids in your life, and give to those who are not.

Woods is a fun and playful child, so when headaches and vomiting became routine, his family rushed to their local hospital. Once tests confirmed brain cancer, Woods' parents sought a referral to St. Jude, where a combination of surgery, chemotherapy and radiation therapy would eventually save his life. Treatments invented at St. Jude have helped push the overall childhood cancer survival rate from 20 percent to more than 80 percent since it opened. We won't stop until no child dies from cancer. "St. Jude means everything to us," said his mom. "They give you hope."

Give today at **stjude.org** | **800-4STJUDE**





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St. Jude Children's
Research Hospital

That “No More Chemo” Glow

In the Kay Research and Care Center, children can change the lighting in their hospital rooms from sunny yellow to jungle green; mandarin orange to purple pizzazz. It's one way kids can exert a little control during a tumultuous time.

Seven-year-old Alexander Bolding discovered that neon items glowed when he switched the lights to electric blue. Then he and his family decided to glow crazy: adding a jolt of color to the traditional No More Chemo celebration.

“We invited his nurses and physical therapists and other hospital friends,” his mom says. “We had glow sticks and decorated the windows. We made everything glow. It was super fun.”

Since January, Alexander has been undergoing treatment for a brain tumor called medulloblastoma. “He’s been a little joyful rock star,” his mom says. Nevertheless, Alexander’s looking forward to the day when he can glow home.



SETH DIXON