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# Promise

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and Elias Aguirre Martinez*  
*Photo by Seth Dixon*



# Cancer in the Family Tree

Some children have genetic conditions that lead to cancer. A new program at St. Jude explores those connections.

**By Ginger Porter**

**T**he engaging smile and clear, blue eyes of Megan Vess belie that her health has ever been in jeopardy. A plastic hospital bracelet offers the only clue—hinting at Megan’s cancer battle and her underlying genetic condition.

Upon receiving a cancer diagnosis in 2012, Megan and her mom had two hours to toss their belongings into suitcases and rush to the airport. At St. Jude Children’s Research Hospital, the 10-year-old immediately began treatment for acute lymphoblastic leukemia.

Genetic evaluation, counseling and testing revealed that an extremely rare genetic condition had made Megan vulnerable to getting this cancer.

Thanks to Megan’s genetic counselor at St. Jude, she

and her family now have a better understanding of how the condition affects her health and her future. A new medical service—the St. Jude Cancer Predisposition Program—has been created for children like Megan, who may have inherited genetic mutations that increase their cancer risk.

“This gene mutation also puts Megan at a higher risk of getting other kinds of cancers,” explains her mom, Amber Vess. “We now understand that she needs screenings and tests to make sure that if she gets another kind of cancer we can find it early.”

## **Kids at risk**

Headed by pediatric oncologist Kim Nichols, MD, the Cancer Predisposition Program evaluates children who have hereditary forms of cancer. The staff also sees kids who do not



have cancer but have genetic conditions or family histories that put them at increased risk for developing it.

Expansion of the program originated with the St. Jude – Washington University Pediatric Cancer Genome Project, an unprecedented effort to sequence the normal and cancer genomes of 700 children with cancer. Now scientists and clinicians at St. Jude are teaming up to apply that information to the clinic.

**5% to 10% of children with cancer have the disease because they carry a predisposing gene mutation.**

“By identifying rare cancer-predisposing genes, we can learn a lot about why tumors form and how to better treat them,” Nichols says.

Eventually, all cancer patients who come through the doors of St. Jude will have the opportunity to have their genomes sequenced.

“It’s estimated that 5 to 10 percent of children with cancer have the disease because they carry a predisposing gene mutation,” Nichols explains. “That percentage may be even higher, because some children carry mutations in genes that have not yet been identified. The only way to learn about these possibilities is by looking at the entire genome of an individual. That’s why the clinical genomics effort is important.

“Identification of these mutations helps the family better understand why their child developed cancer,” Nichols continues. “It also can have an important impact on how you might treat that patient, and it has implications in future reproductive questions and the health of other family members.”

### Examining the family tree

The Cancer Predisposition Program focuses on the whole family, working with patients, siblings and parents.

Licensed certified genetic counselors Emily Quinn and Rose McGee encourage families to find out as much as they can about their medical history, paying

## Helping families manage cancer risk

Staff in the St. Jude Cancer Predisposition Program:

- Discuss information on specific conditions that can increase the risk for cancer
- Provide genetic counseling
- When needed, arrange genetic testing
- Review and explain genetic test results
- Explore family histories to determine possible cancer risks for other family members
- Explain what cancer screening tests are available
- Assist with setting up cancer screening tests
- Share the results of cancer screening tests
- Provide guidance about cancer treatment
- Offer information about taking part in research
- Help families find mental, emotional, social and spiritual support



*Kim Nichols, MD  
Director, Division of  
Cancer Predisposition*

SETH DIXON



**Thirteen-year-old  
Megan Vess is  
learning to  
manage her  
cancer risk.**

**The pioneering expertise and technology  
built by the Pediatric Cancer Genome  
Project make St. Jude the perfect place to  
launch such a program.**

particular attention to family members who have had cancer. From studying the family tree and the child's medical information, the counselors determine if any family members, including future children, might have a higher-than-normal likelihood of developing cancer.

"It's sort of like an onion," McGee says. "The child's at the center, and then you move out to the different layers of the family. First you address the people surrounding the child, and then you move out to the wider family."

The counselors educate the families. If genetic testing is needed, the staff and families discuss the risks, benefits and limitations of those tests. When a genetic diagnosis is made, the clinic staff helps families obtain appropriate medical care as well as mental, emotional, social and spiritual support.



*Licensed Certified  
Genetic Counselor  
Emily Quinn*

"I feel privileged to be able to empower families with knowledge to make informed decisions about whether or not they want to do genetic testing and then—if there's a positive test result—help them to make decisions about their health," Quinn says. "It's an honor to be able to help families process that information and connect them with the appropriate resources."

### **Making important decisions**

Sometimes, genetic findings affect family planning. Megan Vess' mom says McGee gave her the information she and her husband needed to make important decisions.

"We were thinking about having another biological child after Megan got off treatment," Amber says. "Then, Rose explained that the probability of another child getting this gene mutation would be the same risk that Megan had. It helped us to decide that we wouldn't want to take that chance."

The couple has a large and loving family that includes three adopted children.

Megan's genetic mutation means she is susceptible to having other cancers, as well as passing one of her mutated

BETH DIXON



*Megan Vess and her mom, Amber, discuss their family tree with Licensed Certified Genetic Counselor Rose McGee. "Rose helped me understand that this gene mutation puts Megan at a higher risk of other kinds of cancers," Amber says. "That means more screenings and tests to make sure that if Megan gets another kind of cancer we will find it at an early stage."*

**“I feel privileged to be able to empower families with knowledge to make informed decisions about whether or not they want to do genetic testing and then—if there’s a positive test result—help them to make decisions about their health.”**

genes on to any future children. At St. Jude, she has learned about specific cancer screening tests she should have and lifestyle choices she can make to reduce her cancer risk.

Suggested testing includes regular screenings of her digestive tract, brain MRI, blood work, and, when she is older, gynecologic screenings. Megan was relieved to hear she can get many of the tests done at St. Jude.

“St. Jude is Megan’s comfort zone, and to know the screenings could be done there made it so much better,” Amber says.

## Personalizing treatment

The Cancer Predisposition Program, like all facets of St. Jude, addresses the child’s overall health and well-being. Staff members walk families through the process of genetic counseling and testing, offering access to appropriate psychological or child life services, when necessary.

Nichols’ vision is to develop a world-class program at St. Jude. To do that, she plans to expand the team, adding more physicians, counselors, a nurse practitioner and research study staff. Soon, every new St. Jude cancer patient will be offered genetic evaluation and the ability to enroll in clinical trials focused on cancer predisposition.

The pioneering expertise and technology built by the Pediatric Cancer Genome Project make St. Jude the perfect place to launch such a program.

“St. Jude is the only pediatric oncology facility doing this type of analysis to look at the genetic make-up of tumor

and normal cells,” Nichols says. “Genetic technologies are advancing quickly. We’re in an excellent position because we do it better than most other places. We have not done it on a clinical scale before, and it’s time. It’s the way things will be done in the future.”

This personalized approach to cancer treatment allows clinicians to modify treatment based on the genetic make-up of the tumor.

“Therapy can be intensified for those who need it and de-intensified for those who don’t,” Nichols says.

## A whole new world

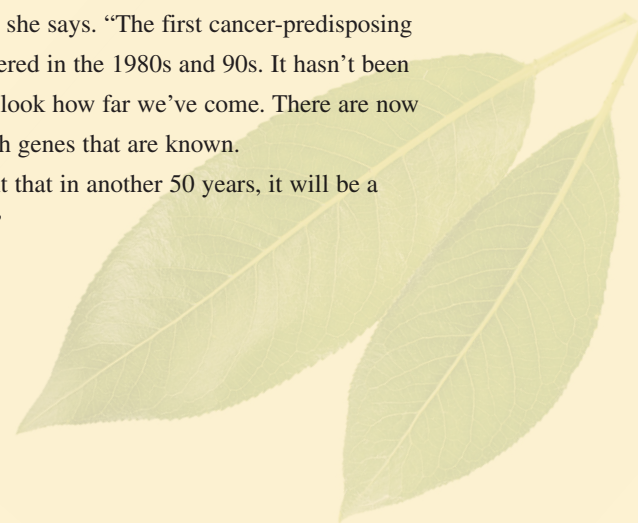
Through the Cancer Predisposition Program, Nichols says, the hospital will receive national recognition for excellence in information, patient management and research participation.

She says her dream is to answer many unanswered questions, such as: Why do some children get cancer and others don’t? What can be done with genetic information to improve cancer treatment or even prevent it from forming?

While curing all childhood cancers is a laudable goal, preventing them from ever occurring would be even better.

“We have a long way to go, but we have come quite a long way already,” she says. “The first cancer-predisposing genes were discovered in the 1980s and 90s. It hasn’t been that long ago, and look how far we’ve come. There are now more than 100 such genes that are known.

“I’m confident that in another 50 years, it will be a whole new world.”





# Teen **A**thletes

By Elizabeth Jane Walker

## at St. Jude: Challenges and Possibilities

How does St. Jude help  
teen athletes  
navigate a new  
and alien  
landscape?





THEY'RE AT THE TOP OF THEIR GAMES: FOOTBALL STARS, BASKETBALL PHENOMS, TRACK STARS, CHEERLEADERS, TEAM CAPTAINS. THEN, IN WHAT SEEMS LIKE A PUNCH TO THE GUT, THE YOUNG ATHLETES ARE SWEEPED INTO A VORTEX OF MEDICAL TESTS AND TERRIFYING DIAGNOSES.

Their identities, dreams and sense of control may take a hit. At St. Jude Children's Research Hospital, staff members help these young athletes grapple with the complex issues that accompany disease diagnosis and treatment.

## Altered identities

Many St. Jude patients previously participated in sports—either on a recreational basis or at a competitive level. But diagnosis and treatment may affect their self-image.

“When teens come to St. Jude, they already have an idea of who they are; what their interests are,” says Jaime Moran of St. Jude Child Life. “Suddenly, so much is out of their control. They feel a loss of normalcy and have left behind friends and activities that they truly love.”

Those feelings often spark fear, angst, uncertainty.

“As an athlete, a huge part of their identity is altered by that diagnosis,” says Jessika Boles, also of Child Life. “People know that person as part of a community of athletes. So the teen has

questions: ‘Who am I going to be now? What are people going to think about me? How are they going to know me? How do I make a name for myself?’

“A lot of what we do in Child Life is help these teens find ways to explore their identity and find other activities that interest them.”

## How much can I handle?

Upon arrival at St. Jude, patients and their parents may initially be timid,



uncertain of how much physical activity they can handle during treatment.

“A lot of times, we must educate our parents and families and let them know it’s OK for the patient to remain active; in fact, it’s better in the long run,” says Angela Corr, DPT, of St. Jude Rehabilitation Services.

### The sky’s the limit

Staff members first help patients define their personal goals.

“It’s all about quality of life,” Corr says. “What is going to make them happy? Then we begin working toward moving them in that direction. With motivation and the right tools, they can often return to doing what they were doing before. If the patient is on board and medically able, then really the sky’s the limit.”

One young man who enjoyed skiing and bicycling was determined to ride a bike after his leg was amputated. He worked with staff in St. Jude Rehabilitation Services to create a plan. Less than two months after surgery, he was back on the bike.

Another teen who was a state bowling champ and basketball player is awaiting surgery to remove a tumor from her leg. Meanwhile, she is participating in a study to evaluate whether physical therapy that occurs before an amputation or a limb-sparing procedure will help her have better function afterward.

Janet Adams, a physical therapist assistant in Rehabilitation Services, says staff must take patients’ interests into account to optimize therapy.

“One of the things that helps motivate our patient athletes is using a creative approach to treatment, incorporating the type of sports they’re involved in and modifying them as needed,” Adams says. “For instance, with a varsity basketball player, we have incorporated basketball into our treatment sessions.”

# My teammate has cancer.

## What now?

### St. Jude staff members offer tips:

- **Keep the patient in the loop.** Most athletes who come to St. Jude say they still want to know details about their team and how the season is progressing.
- **Make an effort to stay in touch.** Let the patient know that he or she is still important to the team.
- **Give the patient an opportunity to be involved.** That could mean cheering from the sidelines, being recognized during a game, or serving as the team videographer or manager.
- **Don’t be afraid to offer options.** The patient can then choose whether or not to participate.
- **Send cards and letters.** Patients love to receive mail. A simple card or note can brighten anyone’s day.
- **Accentuate the positive.** Help the patient focus on the things he or she *can* do, not things that cannot be done.
- **Show your appreciation.** Find ways to let the patient know that he or she is loved, valued and appreciated for more than just athletic talent. Even though that part of the teen’s identity may have changed, the person is still the same teammate, friend and classmate you’ve known in the past.

**“It’s amazing to work with these families. These kids are so resilient and so full of life. While I’m helping them and teaching them, they are teaching me just as much every single day.”**



# The teen has QUESTIONS:

*“Who am I going to be now?”*

*“What are people going to think about me?”*

*“How are they going to know me?”*

*“How do I make a name for myself?”*

## New vistas to explore

Sometimes, goals must be altered. In those cases, St. Jude staff help patients deal with their grief, adjust their expectations and choose new activities that can bring them joy and fulfillment.

“Through consistent, therapeutic presence, we help teens either develop a plan to continue with their sports or we provide opportunities for them to explore other things that are fun or might be physical in a different way,” Moran says.

Help often comes from peers, through relationships honed in one of the hospital’s Teen Rooms—safe environments designed just for them. Through casual conversations and planned events, participants can empathize about losses and challenges, helping one another process changes and possibilities.

“They discuss some really big, heavy-hitting topics,”

Boles observes, “complex questions about emotions and identity and thoughts about treatment and spirituality and connections to self.”

## Measuring success

Often, patient athletes discover exciting new options they could never have envisioned. Some become artists. One former runner began writing a novel—a marathon of a different sort. Some teens demonstrate their athletic skills through playing online sports matches with other patients. One football player became the team videographer for his college team, traveling with them to a bowl game. Cheerleaders who lose their range of motion may learn to make adaptations so that they can still participate.

“I have one patient who played football,” Boles recalls. “He prided himself on being big and bulky. After treatment, he wasn’t able to play football any more, but he returned to weightlifting, because it was something he enjoyed. Now, every time he returns to the hospital for a checkup, he gives me an update on his neck circumference.”

Regardless of the techniques they use to help young athletes, St. Jude staff members agree on one thing: they love seeing patients meet challenges head-on.

“There’s nothing like seeing the happiness on their faces after they meet a goal, even if it’s something as simple as sitting by themselves for five minutes while we play a game,” Corr says. “There’s nothing that can beat that. It’s amazing to work with these families. These kids are so resilient and so full of life. While I’m helping them and teaching them, they are teaching me just as much every single day.” ■



Jessika Boles  
Child Life



Jaime Moran  
Child Life



Janet Adams  
Rehabilitation Services



Angela Corr, DPT  
Rehabilitation Services



# 7 Fitness SECRETS

How do patients maintain the physical and mental fitness necessary for their chosen sports? Several teens share their strategies.

## 1 ■ Take fitness to heart

Matt Dela Cruz had his life under control when his world turned topsy-turvy. The young athlete played volleyball, soccer and ran track. He also played forward for Guam's national basketball team, helping them bring home both gold and bronze medals.

Suddenly, college plans and basketball practices were relegated to the back-burner so that he could begin treatment for acute myeloid leukemia.

To maintain fitness during treatment, Matt works out in the gym at his St. Jude residence, Target House.

"One of the chemotherapy drugs I have taken affects the heart long-term, so I do cardio to strengthen my heart," Matt says. "I do cardio and then lift weights."

Matt says he also gains strength from his clinical team and his peers in the St. Jude Teen Room.

"There's a lot of bad stuff happening in the world," he says. "You want to know where all the good people are? They're all here at St. Jude."



## 2. Set new goals

Before he arrived at St. Jude, high school freshman Zach Fejeran had developed a novel way to develop his upper body strength for football: chopping wood.

"I just like to do it," he says with a laugh.

But in December of 2014, Zach put that activity on hold when he was tackled by Burkitt lymphoma.

Because of the central line implanted in his chest, Zach focuses on other body parts during his regular workouts at Target House. He keeps a log of his progress, with plans for returning to football, as well as participating in shot put and discus during track and field season.

"I've never really been strong in my legs, so I thought this was a good time to build them up," says Zach, whose regular routine includes leg presses and treadmill workouts, as well as bicep curls and tricep exercises.

His advice for other patients hoping to return to competition is short and sweet: "Try to build up again," he says.



SETH DIXON

## from St. Jude Patient Athletes

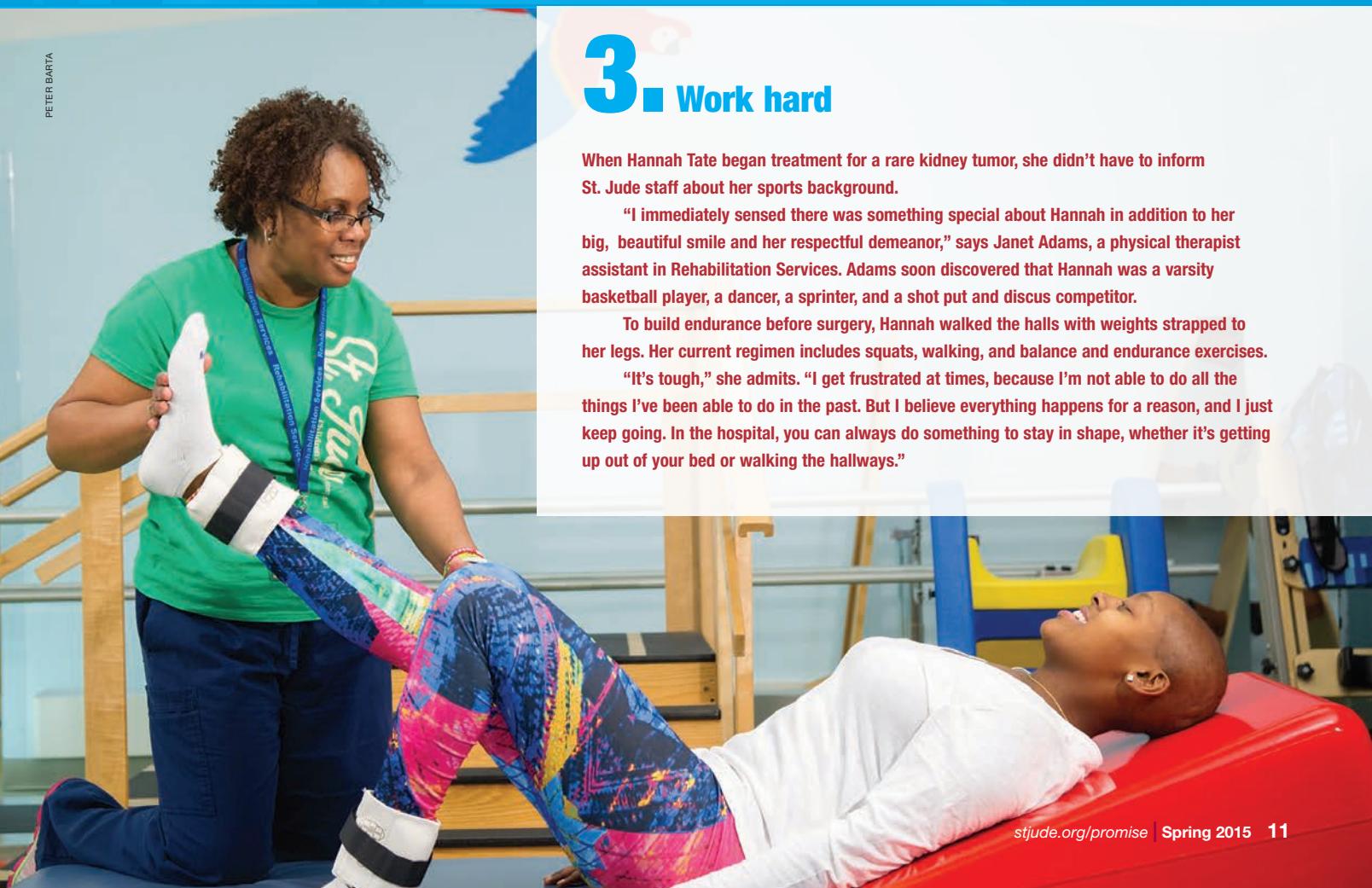
## 3. Work hard

When Hannah Tate began treatment for a rare kidney tumor, she didn't have to inform St. Jude staff about her sports background.

"I immediately sensed there was something special about Hannah in addition to her big, beautiful smile and her respectful demeanor," says Janet Adams, a physical therapist assistant in Rehabilitation Services. Adams soon discovered that Hannah was a varsity basketball player, a dancer, a sprinter, and a shot put and discus competitor.

To build endurance before surgery, Hannah walked the halls with weights strapped to her legs. Her current regimen includes squats, walking, and balance and endurance exercises.

"It's tough," she admits. "I get frustrated at times, because I'm not able to do all the things I've been able to do in the past. But I believe everything happens for a reason, and I just keep going. In the hospital, you can always do something to stay in shape, whether it's getting up out of your bed or walking the hallways."



PETER BARTA





## 4 ■ Get back in the game

It was a freak accident. A lightweight dodgeball hit Jaxon Hindman in the head when he was looking the other way, knocking him to the floor. During a subsequent CT scan, doctors spied an ominous mass: a brain tumor called medulloblastoma. In the previous weeks, Jaxon had experienced no symptoms; in fact, in the days and weeks before his diagnosis, he had run several miles and had zoomed down ski slopes.

The 13-year-old has always led an active lifestyle, including football, baseball, lacrosse and basketball. During treatment, doctors warned that contact sports such as football might not be a good option in the future. So Jaxon is focusing on basketball. Last fall, he insisted on playing the first game of the season in spite of having influenza only days before.

Jaxon credits St. Jude nurse anesthetist John Davidson with providing inspiration during his treatment. "I want to be a CRNA and work at St. Jude and help kids just like Mr. John helped me," he says.

## 5 ■ Follow your passion

Gimme an *H*, give me an *O*, gimme an *RSE*. What does that spell? Therapy!

These days, cheerleader Hannah West doesn't leap off pyramids or run long distances. But she has found a creative way to strengthen her leg, after undergoing surgery to remove a bone tumor called Ewing sarcoma.

To cure her cancer, Hannah received chemotherapy, a bone marrow transplant and radiation therapy. Formerly a competitive cheerleader and dancer, she wanted to return to cheerleading after treatment. St. Jude physical therapists helped her work toward that goal.

Today, she is a member of her school's cheerleading squad and a manager of the basketball team. "I can do pretty much everything that the other girls do," she says.

Hannah has also taken up horseback riding. "The riding strengthens my leg," she explains. "I always wanted to be around horses, and I plan on continuing to ride for the rest of my life. Once I get older, I'm leaning toward either working at St. Jude or running a horse rescue."







## 6 ■ Get a move-on

"Are you ready?" asks St. Jude Physical Therapist Angela Corr, DPT, as Lin Zheng steps onto the treadmill in St. Jude Rehabilitation Services. In response, Lin accelerates his pace. For the next eight minutes, he alternates faster and slower tempos, followed by a session of balance exercises.

The former swimmer and cross country runner is recovering from the effects of a bone marrow transplant he received as part of therapy for acute myeloid leukemia. To increase his stamina and muscle tone, he walks, does squats and lunges and calf exercises. Recently he has begun interspersing running—gradually increasing the duration and intensity.

St. Jude staff encouraged Lin throughout the long process of rebuilding his strength.

"They make you move when you really don't want to," he says, "and they introduced me to exercises that were really helpful."

After experiencing lung problems during transplant, Lin has advice for other teens who want to maximize their progress after transplant: "Don't stay in bed too long," he says.



## 7 ■ Push through and give back

In a hospital filled with children, Nick London draws his share of curious glances. At 6-foot, 5-inches tall, the ninth-grader towers over his medical team. But like many patients at St. Jude, he is engaged in a head-to-head match with cancer.

Already receiving serious attention from college basketball recruiters, Nick has his sights firmly set on the NBA.

His short-term goal is to maintain his fitness during the two-and-a-half year treatment for acute lymphoblastic leukemia. Twice a week, he hits the gym, running, lifting light weights and doing agility training.

While undergoing treatment, he has made a conscious effort to help others—hosting a charity basketball tournament and spearheading a toy drive for the kids of St. Jude.

His message for other patients could apply to most challenges in life: "My advice is to just push through it," he says. "It's not going to be easy, but it can be done. You just have to tell yourself that you can do it."

*Serious about*

# AML

While one little boy dons wacky hats and dreams of four-wheelers and mud pits, scientists and clinicians at St. Jude search for new ways to vanquish high-risk acute myeloid leukemia.

**By Elizabeth Jane Walker**

**A**sk 7-year-old Aiden Walley about his interests, and you're in for an earful. He loves riding four-wheelers. Hunting with his daddy. Playing in the dirt. But for the past few years, he has had to limit those activities in lieu of other pursuits: Traveling to medical appointments. Enduring chemotherapy and radiation treatments. Undergoing three—yes, three—bone marrow transplants.

This rough-and-tumble boy with an ever-present smile and a silly disposition has been locked in a battle with an aggressive form of acute myeloid leukemia (AML).

In January of 2013—just four months after Aiden completed his initial treatment—the leukemia returned with a vengeance. The cancer persisted through two transplants, the second using blood stem cells from his mom. In October of 2014, Aiden had a third transplant. This time, his immune system was wiped out and replaced with stem cells from his grandmother.

“It’s hard to understand how some leukemias can survive chemotherapy, radiation therapy and a new immune system,” says oncologist Jeffrey Rubnitz, MD, PhD, of St. Jude Children’s Research Hospital. “When leukemia survives transplant, you know it’s extremely resistant to treatment.”

Inspired by St. Jude patients like Aiden, Rubnitz and

his colleagues are working to find a cure for AML that has either failed to go into remission after treatment or has returned after therapy.

## **A novel approach**

AML is generally more difficult to treat than the most common childhood cancer, acute lymphoblastic leukemia (ALL). The survival rates reflect that dichotomy. AML has a 70 percent survival, compared to 94 percent for ALL. About 30 percent of children with AML relapse or have disease that is refractory (resistant to treatment). For those children, the prognosis is grim.

Before his third transplant, Aiden took part in a clinical trial designed to find out if a new drug called selinexor is safe and if it can benefit young AML patients. Selinexor is combined with two chemotherapy drugs with equally cumbersome names: fludarabine and cytarabine.

“Selinexor works by a mechanism that is totally different than any other drug used for cancer,” Rubnitz says.

Cancer is caused by the uncontrolled growth of cells. One way these cells flourish is by getting rid of tumor suppressor proteins—molecules that would normally cause cancer cells to die. Selinexor traps those proteins inside the nucleus of each cell, which may cause the cancer cell to stop growing or die.

“This drug restores the leukemia cell to a more normal state, which, we hope, will make those cells more susceptible





To look at Aiden Walley—with his constellation of freckles and silly hats—it's hard to fathom that this 7-year-old is locked in a battle with an aggressive case of acute myeloid leukemia.



to standard chemotherapy,” Rubnitz explains.

The triple-drug combo is one of three new approaches developed at St. Jude for children with high-risk AML.

## Taking aim at a mutated gene

A second clinical trial is designed for children who have a mutation, or change, in a gene called *FLT3*. Ten to 15 percent of children with AML have this mutation, which helps leukemia cells thwart the effects of standard AML chemotherapy.

“This mutation makes leukemia cells grow rapidly,” explains St. Jude oncologist Hiroto Inaba, MD, PhD.

“For years, we gave chemo, chemo, chemo, and the disease would come back even after transplant,” Inaba says. “In our previous St. Jude protocol, we found that a drug called sorafenib is effective against leukemia cells that have this mutation.”

However, six months to a year after taking sorafenib,

**“The mutations would sneak up.”  
Something had to be done.**

children with the *FLT3* mutation would often acquire more genetic changes.

“The mutations would sneak up,” Inaba says.

Something had to be done.

Inaba teamed with Sharyn Baker, PharmD, PhD, and Eric Zimmerman, PhD, both of St. Jude Pharmaceutical Sciences. They found that an experimental drug called crenolanib prevents the growth and survival of AML cells with the mutation. Scientists suspect that crenolanib also blocks leukemia cells that acquire additional mutations after exposure to sorafenib.

Crenolanib has been used to treat pediatric brain tumors, but it has never before been used against pediatric AML. The current clinical trial combines crenolanib with sorafenib to deliver what Inaba and his colleagues hope is a one-two punch to leukemia in children with the *FLT3* mutation.

SETH DIXON



Jeffrey Rubnitz, MD, PhD, of St. Jude Oncology, talks with John Michael Martinez, as the 11-year-old prepares for his third transplant. John Michael took part in a new clinical trial designed to find out if a drug called selinexor is safe and if it can benefit patients with AML.

PETER BARTIA



Tanja Gruber, MD, PhD, of St. Jude Oncology is developing a clinical trial for relapsed AML. In the lab, she discovered a drug combo that is effective for AML patients with rearrangements of the MLL gene.

SETH DIXON



Hiroto Inaba, MD, PhD (at left), of St. Jude Oncology has teamed up with Eric Zimmerman, PhD, and Sharyn Baker, PharmD, PhD, of Pharmaceutical Sciences to develop new options for children with AML who have a mutation in the *FLT3* gene.



## New options on the horizon

Two new options are in the works for children with relapsed or refractory AML.

One clinical trial involves a process called micro-transplantation, in which chemotherapy is followed by an infusion of peripheral blood stem cells from a donor.

“When the patient mounts an immune response to reject those stem cells, the activation of the immune system may attack the leukemia cells, as well,” Rubnitz says. “So you’re activating the patient’s own immune system as well as the donor’s immune system. It seems to have a very potent anti-leukemic effect.”

Research indicates that response rates to micro-transplantation are good, the toxicity is low, and the patients rebound more quickly than in regimens that include chemotherapy alone.

“We’re very excited about it,” Rubnitz says.

Tanja Gruber, MD, PhD, of St. Jude Oncology is also working on a clinical trial for relapsed AML based on research in her lab. Gruber’s work suggests that, when given along with chemotherapy, two drugs called bortezomib and vorinostat are effective for AML patients who have rearrangements of a gene called *MLL*.

“It will be nice to have four options for patients with relapsed AML,” Rubnitz says. He emphasizes, however, that stem cell transplantation remains the gold standard for treating relapsed AML.

“Our goal in all of these therapies is to safely get these patients into remission so they can then receive stem cell transplants and be cured,” Rubnitz says. “In general, the more

**“We want to get these patients to transplant safely with very low levels of disease.”**

leukemia a child has going into transplant, the higher the chance of relapse afterward. So we want to get these patients to transplant safely with very low levels of disease.”

## Seize the day

After nearly three years at St. Jude, Aiden continues his battle with AML.

The most challenging part of their journey has not been the distance from home—though it has been difficult. It has not been the treatment—though the therapy has been grueling at times.

“The most difficult thing you go through,” Anna says, “is just knowing your child has cancer. If Aiden weren’t bald, you’d probably never know he had cancer. He tires out now more than he did in the beginning, but if you give him a nap and he catches his second wind, he’s good to go.

“You have to just take it as you go,” she continues. “You take your good days and run with ’em.” ■



*After nearly three years at St. Jude, Aiden continues his battle with AML.*



# St. Jude on the Phone

Thanks to **St. Jude support**, long-term cancer survivors obtain the health screenings they need.

By Anita L. Houk

Nurse practitioners Brenda Steen (pictured) and Susan Ogg helped survivors obtain screenings.

**C**raig Wight is 54 and living life with gusto: staying fit, eating well and getting regular medical checkups.

Health complaints? Few.

So few, in fact, that until St. Jude researchers contacted him about the need to get heart testing, he had not told his primary physician of his early encounter with cancer.

Osteosarcoma.

“I was not meaning to be secretive,” Wight explains, “but it was so long ago that I didn’t think it was all that important.”

He was diagnosed at 17. In people younger than 20, osteosarcoma is the most common type of bone cancer. It typically starts in the arms, legs or pelvis, can spread quickly, and annually affects about 400 patients in the United States and 300 in Canada, Wight’s home.

“My whole knee and about six inches of my femur had to be removed,” he says, “and they replaced that with a metal joint. I had no radiation, but I did have chemotherapy.

“Of course, that was a lifetime ago.”

Thirty-eight years and three metal knees, to be exact.

While Wight for years has complied with requests to

further medical science in ways ranging from completing lengthy surveys to collecting his DNA, he knew little of the need for his own special heart screening, called an echocardiogram.

He also did not realize that some childhood cancer survivors are at increased risk for certain heart problems.

The takeaway: Get informed; get an echocardiogram.

## Recognizing risks

St. Jude Children’s Research Hospital staff members who contacted Wight and other survivors were working on how best to get that message out, awareness levels up and survivor screenings completed. Their study, published in the *Journal of Clinical Oncology*, used two techniques to try to motivate 472 survivors to get echocardiograms:

- One group was mailed a personalized health care plan.
- A second group received the printed plan plus telephone counseling.

The study measured, among other things, how many patients in each group overcame obstacles—such as personal fear, insurance issues or physician reluctance—



and, within a year, got the echocardiograms.

The phoned group won, hands down.

“Specific treatments contribute to a higher risk of heart disease in our survivors as they age,” explains Melissa Hudson, MD, director of the St. Jude Cancer Survivorship Division. She and Cheryl Cox, PhD, of St. Jude Epidemiology and Cancer Control, headed the project, which was part of the Childhood Cancer Survivor Study (CCSS). St. Jude coordinates the CCSS, which tracks the health of more than 20,000 survivors of childhood cancer who were treated at St. Jude and other medical centers throughout the U.S. and Canada.

“We want survivors to be aware of the potential risks for, in this case, cardiomyopathy, a heart muscle weakening,” Hudson says.

## The heart of the matter

Periodic screening with an echocardiogram—a test that uses sound waves to create detailed pictures of the heart—can detect problems early and enhance interventions to preserve heart function.

However, getting the test at the recommended times depends on patient awareness, vigilance and, often, persistence, as patients face barriers.

The phone interventions, Hudson says, were tailored to each survivor’s questionnaire responses and subsequent conversations with specially trained, advanced-practice nurses.

“Some people call it motivational interviewing,” says Cox, who specializes in the role of motivation in health behavior.

Cox developed the technique and trained St. Jude nurse practitioners Susan Ogg and Brenda Steen to use it.

“It’s showing obvious respect for a patient, obvious concern, not being authoritative in language or approach. The patients are in control of their destinies. We are there to provide information,” Cox says.

For Wight, that approach worked.

“Susan and Brenda were great,” he says. “The phone call definitely convinced me that I should get the echocardiogram. It was good-natured insistence. Also, my wife gave a little push.”

“Getting participants’ trust was key,” Steen says. “We had two telephone conversations with them. The first was

the most important. The patients were finding out new information.

“We gave them the information, talked them through it, helped them follow up and gave them the resources they needed to obtain the echocardiogram,” Steen says.

## The personal touch

Obstacles to getting the tests varied. Some patients did not recognize the need or believe it was important. Others said they could not afford the test because of high insurance deductibles, limited coverage or no insurance at all. Some patients said they didn’t have time or simply forgot.

Some of the patients’ doctors did not understand or, perhaps, agree with the recommendation; refused to order the test; or ordered electrocardiograms, which check the heart’s electrical activity, instead of the echocardiograms that were required.

To urge compliance, researchers contacted doctors and wrote insurance companies to explain the patients’ needs.

The study found that the human connection—using trained advanced-practice nurse phone counselors—more than doubled the percentage of patients, ages 25 to 59, who got the needed echocardiograms. Of the printed plan-plus-counseling group, more than half got tested, compared with 22 percent of the printed plan-only group.

Of the total 472 participants, 153 got echocardiograms. Some of those tests revealed heart problems requiring treatment or medical monitoring.

Wight, pleased that his echocardiogram provided a solid baseline with no abnormalities, understands the survivor fear that goes with medical testing.

“Cancer is a big deal in your life,” he admits, “and there’s always anxiety when you go to the doctor.”

So, would he have gotten the echocardiogram if ...

“If I’d received just the printed material? I doubt it.” ■



*Melissa Hudson, MD, director,  
St. Jude Cancer Survivorship*



*Cheryl Cox, PhD, St. Jude  
Epidemiology and Cancer Control*

# TEACHING by EXAMPLE

The De Groot Family Foundation  
fulfills one couple's legacy.

By Rachel Schwartzberg

Peter and Louise De Groot achieved the American dream, and hand-in-hand with their success was the desire to give back to the country that welcomed them.

Arriving in the United States from the Netherlands in the 1950s, the De Groots became successful through hard work and determination. Today, their legacy lives on through the De Groot Family Foundation.

Peter started a wholesale nursery business, De Groot Inc., in 1957 near their home in southwest Michigan. While helping out in the business as youngsters, the couple's three children inherited their father's strong work ethic and unwavering values.

"My parents believed in education and saw to it that their children and grandchildren had that opportunity," says the De Groots' oldest daughter, Shirley Leith. "They felt it was the key to getting places in life."

The couple also regularly gave to charity.

"They did it because they believed it was the right thing to do," Shirley says, "and they never wanted any recognition."

After the De Groots' son, Ron, lost his battle with cancer in 1985 at the age of 27, the family began to support cancer charities, including St. Jude Children's Research Hospital.

"St. Jude has become so important to us," Shirley explains. "The year following my brother's death, my brother-in-law died of brain cancer. It was a difficult time."

The family named a diagnostic imaging recovery bay in the St. Jude Chili's Care Center in tribute to of Ron De Groot and Christopher Leith. The plaque gently reminds patient families and employees to "Believe in Miracles."

Shirley and her mother established the De Groot Family Foundation to streamline the family's charitable giving after Peter's death in the mid-1990s. Mother and daughter served on the foundation's board of directors together with the family's attorney, a close family friend.

Louise lived to see the next generation of her family share in her devotion to charity when Shirley's two children, Jessica and Eric Leith,

joined the foundation. Shortly before Louise's death in 2013, the De Groot Family Foundation made a five-year commitment to support the St. Jude Brain Tumor Program.

Although Shirley has not yet visited the hospital in person, her daughter, Jessica, had the opportunity to tour the facility.

"She felt it was an amazing place," Shirley shares. "Her reaction was, 'If a child has to be sick, what a wonderful place to be.'"

Since retiring from the family business, Shirley and her husband, Mike, frequently travel to Seattle to visit Jessica and their baby granddaughter.

"Our kids are our greatest pride and joy," Shirley says. "I hope they will carry on the foundation. They have become a big part of it. I'm especially proud of them for continuing the legacy that my parents taught by example." ■

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**"If a child  
has to be  
sick, what  
a wonderful  
place to be."**

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The work begun by Louise and Peter De Groot (top photo) continues, thanks to the beneficence of (bottom photo, from left) Shirley Leith and her children, Eric and Jessica.



# A HERO AMONG US

With each footstep,  
**Kyllian Warman**  
honors her father  
while helping save  
children's lives.

By Leigh Ann Roman

**K**yllian Warman of New Jersey has transformed the challenges in her life into an opportunity to be a hero for the children of St. Jude Children's Research Hospital.

The 20-year-old college student has run 12 full marathons since April 2013, raising more than \$60,000 through the St. Jude Heroes program. As a result, she was recognized with the Heroes Among Us Award during the 2014 St. Jude Memphis Marathon Weekend.

She began running as a way to deal with tragedy in her own family. When she was a high school freshman, her father, Rick, was found to have colon and liver cancer. Always an athlete, Kyllian added running track as a form of therapy, becoming successful in the sport.

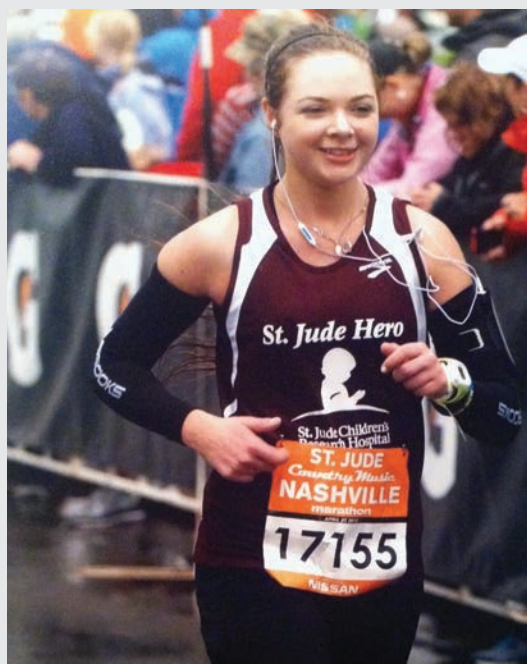
In 2012, her father's condition worsened. Kyllian decided to run 2,012 miles that year—reaching that goal by making her daily 16-mile roundtrip school commute on foot. The long runs helped her process her feelings.

**“Everyone should be a hero  
once in their life.”**

“I just needed to clear my head,” she explains.

Her father died the day of Kyllian's high school graduation, holding her hand after she returned home from the ceremony.

“I wanted to keep running, but to run for something much bigger than myself,” Kyllian says.



*“I wanted to keep running, but to run for something much bigger than myself,” Kyllian Warman says.*

“I wanted to do it to benefit cancer research.”

Kyllian found the St. Jude Heroes program, which allows runners and walkers to raise funds by seeking pledges for participation in endurance events.

St. Jude is the sole beneficiary of the St. Jude Memphis Marathon Weekend and the St. Jude Country Music Marathon in Nashville, Tennessee. Charity athletes can also raise funds as St. Jude Heroes by taking part in other marathons and half marathons nationwide. Fundraising through the program has grown from \$2.3 million in 2008 to \$11.9 million in 2014.

A year after her father died, Kyllian ran her first marathon in Nashville. The following year, she was the top St. Jude Hero fundraiser for that event, bringing in \$25,000—while working and attending college full time.

Kyllian regularly speaks about St. Jude at events in her New Jersey

hometown. After one such talk, a prominent businessman introduced himself and said he was going to write a \$10,000 check for St. Jude.

“I fell to my knees and started crying,” she says. “I realized just how big the support had grown.”

Kyllian says the St. Jude Heroes program appeals to so many people because it is positive on every level.

“Who doesn't want to get excited about how good their life is and who doesn't want to help these kids have a better life?” she says. “For me, the Heroes program has really transformed everything. Everyone should be a hero once in their life.”

**FIND OUT how to become a St. Jude Hero: [stjudeheroes.org](http://stjudeheroes.org) ■**

# Research Highlights

## Question yields strategy for tropical disease



Researchers like to ask basic questions about the world and how things in it work. Answers to those questions can advance science and protect health.

A recent example comes from the St. Jude lab of Thirumala-Devi Kanneganti, PhD, Immunology (pictured at left with Prajwal Gurung, PhD, Immunology). Scientists wanted to know more about how one branch of the disease-

fighting immune system influences another in response to parasite infections. The researchers wound up discovering a possible strategy for fighting the most common form of a major tropical disease.

The disease is cutaneous leishmaniasis. It is caused by a parasite that spreads through the bite of infected sand flies. The infection causes skin sores that sometimes take years to heal. The results can lead to scarring and disability. Each year leishmaniasis kills thousands of people in some of the world's poorest countries and sickens more than 1.3 million. In many countries, children are particularly vulnerable.

Researchers showed that blocking a protein named interleukin 18 (IL-18) protected specially bred mice from the infection. The results suggest that developing drugs that neutralize IL-18 could lead to better prevention and treatment of the disease. This research appeared in the *Journal of Clinical Investigation*.

## Drug candidate destroys malaria in 48 hours

Malaria is a serious health threat to billions of people around the world, particularly children. The World Health Organization estimates that a child in Africa dies of malaria every minute. Although malaria can be cured, the parasites that cause it often develop drug resistance, complicating efforts to fight the disease.

Now, a fast-acting drug candidate developed by St. Jude scientists and collaborators offers new hope for beating malaria. The candidate, called (+)-SJ733, triggers the immune system to rapidly destroy red blood cells infected by the deadliest type of malaria parasite. After only 48 hours, the parasites are gone without a trace.

Clinical trials are being planned to test the safety of (+)-SJ733 in humans. "Our goal is to develop an affordable, fast-acting combination therapy that cures malaria with a single dose," said R. Kiplin Guy, PhD, chair of St. Jude Chemical Biology and Therapeutics.

"These results indicate that SJ733, and other compounds that act in a similar fashion, are highly attractive additions to the global malaria eradication campaign, which would mean so much for the world's children."

The work was published in the *Proceedings of the National Academy of Sciences*.



## Scientists zero-in on monocytes

The body's immune system fights cancer. And cancer fights back. For years, researchers have known that tumors can harness healthy white blood cells to enhance their own growth and suppress attacks by the immune system. But it was unclear exactly which types of white blood cells help tumors.

The puzzle has been solved by researchers from St. Jude, who used a novel strategy to track the contribution of different types of white blood cells. They discovered that the immune suppression is primarily the work of cells called monocytes.

The laboratory findings mark a turning point in cancer

immunology and provide the foundation for developing more effective immunotherapies, said Peter Murray, PhD, of St. Jude Infectious Diseases and Immunology.

"We have identified the monocytic cells as the important cell to target—not only in cancer, but possibly for treatment of autoimmune disorders like rheumatoid arthritis or inflammatory bowel diseases where dampening the immune response could provide relief," he said.

The results were published in the scientific journal *Immunity*.

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## Add pituitary tests to survivor health checklist

St. Jude researchers have reported more evidence that many childhood cancer survivors need their pituitary function checked annually.

Investigators found that survivors remain at risk for pituitary hormone deficiencies decades after they underwent treatment with cranial irradiation. Researchers also found evidence that low hormone levels may diminish survivors' health and quality of life.

"The findings underscore the need for childhood cancer

survivors to get recommended health screenings and challenge us to help survivors navigate the health care system and receive the care they need," said Wassim Chemaitilly, MD, of St. Jude Pediatric Medicine. St. Jude is testing a pilot program to help cancer survivors at risk for hormone problems make a successful transition from pediatric to adult medical care.

The study appeared in the *Journal of Clinical Oncology*.

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## Taking aim at treatment-related hearing loss

St. Jude scientists have discovered inherited genetic variations that are associated with rapid hearing loss in young cancer patients treated with the drug cisplatin.

The drug is a mainstay of treatment for children and adults with many types of brain and other solid tumors. But in some patients, the drug causes severe hearing loss or other side effects.

Jun J. Yang, PhD, of St. Jude Pharmaceutical Sciences, and his colleagues checked the DNA of children with brain tumors for more than 1.7 million genetic variations. Variations in a gene named *ACYP2* were associated with as much as a four-fold

greater risk of cisplatin-related hearing loss.

The screening is among the first to survey the genetic landscape for clues to help explain why the risk of cisplatin-related hearing loss varies so widely among patients.

"This is an important first step in being able to pinpoint patients who are at higher risk of developing cisplatin toxicity and to learn how to better manage that risk," said Clinton Stewart, PharmD, of St. Jude Pharmaceutical Sciences.

A report on the study appeared in the scientific journal *Nature Genetics*.



# A Message to My St. Jude Family

A former St. Jude patient comes full circle—giving back as a volunteer.

**By Anthony Maranise**

**R**ecently, in my service as a volunteer Eucharistic lay minister at St. Jude Children's Research Hospital, I was called to visit a little boy with acute lymphoblastic leukemia (ALL). When I went to his room, I saw the all-too-familiar sickness that is a side effect of chemo. His family seemed distraught, having only recently learned of his diagnosis.

In the course of our conversation, I told them, "I've been where you are now. I was a patient here myself not too long ago. We are all family and are all in this together."

The mother began to cry as she hugged me. "Thank you so much," she said. "You have no idea how comforting that is to hear."

Later, she asked: "What was your diagnosis?"

I replied, "The exact same as your son's, and here I am 17 years later!"

On October 11, 1994, I first came to St. Jude as a child with ALL. Every Tuesday for the next two-and-a-half years, I returned for what seemed like countless "sticks," "spinals," chemotherapy doses and exams. In April of 1997, my remission was confirmed. I had battled, beaten and survived the very cancer that once was almost a certain death sentence.

*"To all my St. Jude family, thank you for giving me my second chance at life," Anthony Maranise says.*

Thanks to what I call "the living saints" of St. Jude—the patients, their families, doctors, researchers, nurses, staff, volunteers and generous financial supporters—the form of leukemia I once battled is now nearly beaten for good.

After years of returning to St. Jude for continual monitoring, I "graduated" into the hospital's follow-up program. Today, I return often to St. Jude as a volunteer, bringing spiritual care, hope and comfort to other patients who share my faith.

St. Jude will always be a part of my life. In fact, I even had the logo tattooed on my ankle. It reminds me to be thankful for the people and the place that let me live and to pray continuously for those in my St. Jude family who are presently fighting.

As I continue my graduate studies in theology, preparing to become a priest, I am sometimes asked questions. My favorite question is: "Where is the holiest place you've ever visited?" Hands down, because of the ways in which faith, hope and love are constantly demonstrated in the care for all patients, and through the bravery, perseverance and trusting confidence of all the patients, my answer is always, "St. Jude Children's Research Hospital."

To all my St. Jude family, thank you for giving me my second chance at life. It's my turn now to give back to you. Though I can never entirely repay you all for what you've done for me, I shall pray that my service—both to the children and families of St. Jude and to all of God's children—is a start. ■





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how our gift is  
**helping the  
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—Carol Di Lorenzo

**Their gift gives back.**

*St. Jude patient Charlee  
age 3  
myelodysplastic syndrome*



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## Something to smile about

Employees surround St. Jude President and Chief Executive Officer James R. Downing, MD, during a “silliness break” to celebrate the announcement that St. Jude is listed for the fifth consecutive year as one of FORTUNE magazine’s “100 Best Companies to Work For.”

“From the lab to the clinic to the front desk, everyone at St. Jude plays a role in fulfilling our incredible mission of finding cures for children with cancer and other life-threatening diseases,” Downing said. “When people find meaning in their work and are supported by an environment that fosters teamwork, creativity and compassion, the extraordinary is possible.”



*Finding cures. Saving children.*