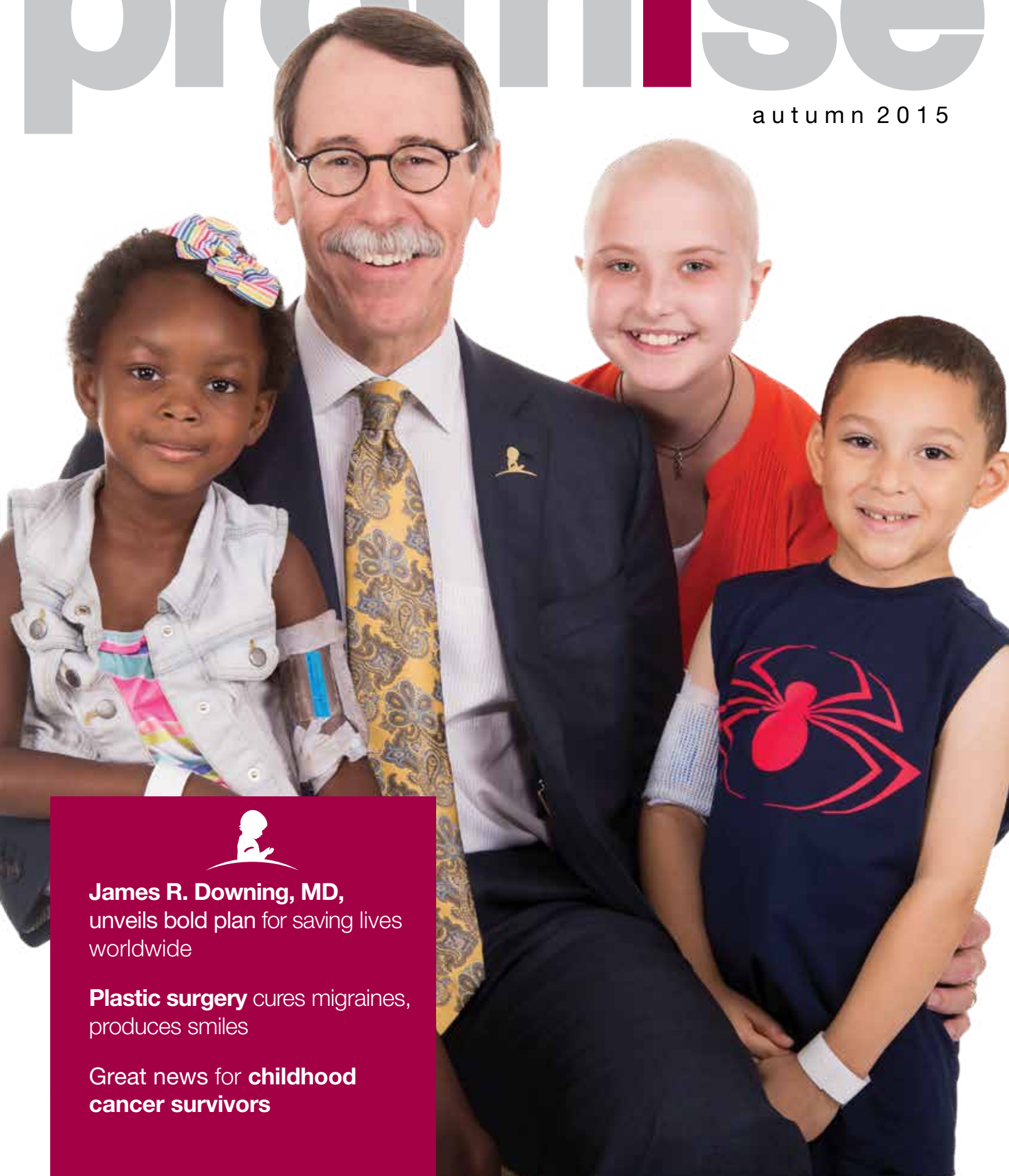


St. Jude promise

autumn 2015



James R. Downing, MD,
unveils bold plan for saving lives
worldwide

Plastic surgery cures migraines,
produces smiles

Great news for **childhood
cancer survivors**



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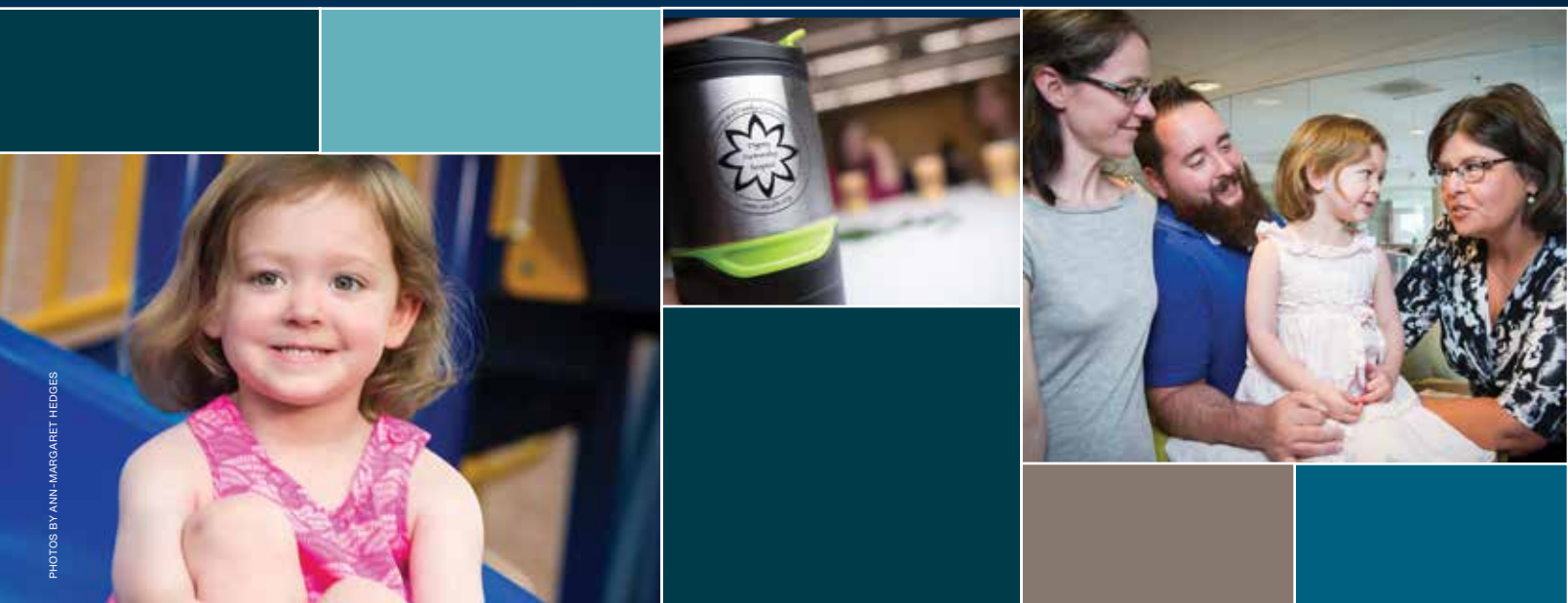
On the cover:

From left: Michelle Longstreet;
James R. Downing, MD; Juliana
Judge; Andrew Frankenberger
Photo by Peter Barta

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WELCOME TO THE FAMILY

Patient family-centered care establishes a partnership among family members and hospital staff.

By Chris Lewis

“The test results show...”

From the moment parents hear these words, their emotions run the gamut—confusion, anguish, fear, even a sense of isolation. But then they meet their medical team at St. Jude Children’s Research Hospital, and they realize they are not alone.

Rather than being treated as hospital guests, parents and other family members are immediately placed at the center of the decision-making process. This is the essence of the St. Jude Patient Family-Centered Care program—a collaborative partnership among family members, doctors and staff that ensures a child’s needs are fully met.

“From the beginning, the hospital has been involved with and focused on the family,” says Alicia Huettel, RN, director of St. Jude Family-Centered Care. “But we’ve enhanced that relationship by using families as advisers in many areas of the hospital. We understand the value that partnering with family members on different levels can provide.”

Coffee, conversation and bonding

Through the St. Jude Family Advisory Council, patients and families make creative suggestions and innovative proposals that have far-reaching effects. This group consists of St. Jude family members as well as hospital faculty and staff.

Katie Witsoe has been a member of the Family Advisory Council since 2012, two years after her 5-year-old son, Sean, passed away from a type of brain tumor called medulloblastoma.

Last year she and other council members discussed ways to solicit opinions and ideas about patient care from parents whose children were inpatients.

In response, the council hosted its first Coffee Talk meeting, an informal parent-to-parent social break. During Coffee Talk’s first session, council members learned that parents were interested in discussing their thoughts about the hospital and were eager to meet other parents.

"Going from appointment to appointment, parents really don't have time to just sit and talk and get to know each other," Witsoe says. "Coffee Talk gives families an outlet to cope as they meet other families and share their experiences. It really builds a bond."

On average, 10 parents attend the meetings, alongside three or four Family Advisory Council family members and at least two St. Jude staff members.

"As a council member, I'm able to give back and turn something that was tragic for our family into hopefully something positive that will help other families as they move forward," Witsoe says. "It's an honor to be on the committee."

Promoting self-advocacy

As one of the pillars of the program, the Family Advisory Council meets regularly to develop opportunities for family members to become involved with the hospital and to discuss ways in which family-centered care can be improved. The council also encourages staff to solicit patient and family input on proposals related to patient-care services.

St. Jude patient Erin Haskins made one such proposal, an

idea that will soon benefit patients throughout the hospital.

For nearly 10 years, Haskins has struggled with the side effects of a brain tumor known as a craniopharyngioma. Sometimes those issues required a visit to her local hospital.

"If I were sick and unable to communicate, my parents wouldn't always know which medications I was taking, and they couldn't share that information with staff members," Haskins says. "So I made a laminated plastic card about the size of a dollar bill. It has my medications, administration times and dosages on the front, and allergies and emergency contact information on the back."

Doctors and nurses at her community hospital were so pleased with the card that Haskins, an aspiring graphic designer, wanted to develop the card for other St. Jude patients. She shared her concept with Huettel, who worked with her on the project's details.

"St. Jude staff are really open to patient and family input to make the hospital a better place," she says.

By the end of 2015, Haskins anticipates that interested St. Jude patients will be able to request MedMinder cards. Haskins will design, print and laminate the cards and mail them



PETER BARTA

Facing page: Alicia Huettel, director of St. Jude Family-Centered Care (at right) confers with Skyler Nolan and her parents, Samantha and Blake. *Above:* Enjoying coffee and conversation are St. Jude parents (from left) Jeanne Erickson, Jessica López, Candice Reed, Debbie Higgins and Sandy Hess.



Longtime St. Jude patient Erin Haskins worked with families and staff to create MedMinder cards, which provide patients' medication details, allergies and emergency contact information at a glance.

to the patients free of charge.

"It's truly a form of patient self-advocacy—being aware of the medications you're on and being able to tell people about it," Haskins says. "I hope these cards can be used by patients throughout the whole hospital. That's my goal."

Embedded construction consultant

Five years after Chris Bridges' daughter Khirsten was diagnosed with sickle cell disease, another one of his daughters, Kaitlyn, received the same diagnosis. One day, as he donated platelets at St. Jude, Chris met a Family Advisory Council member, who encouraged him to attend a meeting.

After becoming acquainted with several council members, Bridges eventually decided to join. About three years ago, he became an integral part of the hospital's design and construction projects, even though he had no prior experience

in either capacity. In that role, Bridges attended project meetings to provide input on new inpatient and parent room construction.

"My interaction with the design and construction projects is parent representation," he says. "From a parent's perspective, what's best for them and the patients? What do the parents need that they didn't have before? And what might help make their stay more accommodating—with the care and comfort of the patient and family in mind?"

Bridges recommends other parents become involved with the council, as well.

"Parents at St. Jude have different perspectives and come from different walks of life, so to have them come together and share their unique opinions on the hospital will not only help St. Jude, but will also help parents and children, in the long-term," he says.



In his role as an embedded consultant for hospital construction projects, St. Jude parent Chris Bridges (*at right*) offers his perspective on construction plans to John Curran, director of Design and Construction.

Making connections

Thus far, more than 200 current and former patients, families and caregivers have joined an online advisory council known as the Family-Centered Care E-Council.

“Some parents or family members are not in a position to join the Family Advisory Council, so this allows them to share information with one another, discuss various topics through a private, online forum and offer suggestions to staff members,” Huettel says. “It’s a way for us to be as collaborative and inclusive as possible.”

Aside from the E-Council, Huettel also manages the Linda R. Hajar Family Resource Center at St. Jude, which offers access to computers, CDs, books, online medical journals and other resources. The center is open 24 hours a day, seven days a week. “It’s really about helping families stay connected with each other. And because it’s open all the time, they can use the center as a place for respite,” Huettel adds.

Blending perspectives

The common purpose of each of these initiatives is to create partnerships among loved ones and medical professionals to improve patients’ quality of care.

“Family-centered care is about partnering with patients and families,” Huettel says.

“St. Jude provides the medical expertise,” she continues. “It’s the blending of those perspectives and that knowledge that provides the best care.”

Bridges says the hospital’s environment also fosters positive patient experiences.

“St. Jude is like a home-away-from-home, and doctors’ and staff members’ hearts and minds are dedicated to families and patients,” he says. “I feel like St. Jude really is a family, and everyone involved is focused on taking care of our children.” ■

Creating Smiles

By Ginger Porter



A new plastic surgery technique cured the migraines of brain tumor survivor Alex Dedinsky.

At St. Jude, plastic surgeons help children regain their quality of life through new techniques ranging from migraine relief to facial reanimation.

At age 7, a brain tumor nearly stole Alexandra “Alex” Dedinsky’s sight. As a teen, it robbed her of a normal life, crushing her with migraines so severe and of such duration as to keep her homebound. Alex was desperate for relief when a plastic surgeon from St. Jude Children’s Research Hospital used an unusual surgical technique to short-circuit the pain. The result left Alex pain free for the first time in years.

St. Jude has expanded its services in plastic surgery, particularly for children who have nerve damage caused by brain tumors or their treatment. For many patients—such as

children with facial paralysis—the service literally gives them a reason to smile.

“We see patients every week and help with the wound care clinic as well,” explains Robert Wallace, MD, chief of plastic surgery at St. Jude. “It’s unusual for a cancer hospital to offer services as comprehensive as these.”

Seeking solace

About 11 years ago, Alex’s parents, both physicians, noticed a problem with her eyesight. In the backyard one day, her dad administered an impromptu eye test, which indicated that Alex had no vision in her left eye and partial vision in the right.

“It had always been that way, and I thought that was the way everybody saw. I didn’t know any differently,” Alex says.

An MRI revealed a tumor pressing on her optic nerve. Tests revealed juvenile pilocytic astrocytoma, a slow-growing tumor of the brain or spinal cord.

A quick survey of the couple's medical colleagues pointed the family to St. Jude. After treatment with radiation therapy, Alex's vision returned, although she does have some lingering problems with her eyesight.

For years, chemotherapy kept the tumor at bay but left Alex coping with headaches.

"I have had headaches since I was little," Alex says. "But, about a year ago, I started getting severe migraines. Nothing helped. For a five-month period, I couldn't do anything. I couldn't go to school. I couldn't read. I couldn't sleep. It really changed my life."

Putting quality into life

After exhausting several treatment options, Alex's St. Jude neurologist, Zsila Sadighi, MD, consulted Wallace. Swelling in Alex's facial muscles and tissues was constricting the nerves in that region, causing chronic migraines. The clinicians used the drug Botox® to relax those muscles, offering temporary relief.

When Alex responded favorably to that treatment, Wallace suspected that she might have an even more dramatic and permanent response with a surgical approach.

He and his associate, Petros Konofaos, MD, devised a plan.

"We incorporated one of the cosmetic techniques we use to do brow lifting, but instead of lifting the brow, we decompressed the nerves in that region. It's a new technique. This was the first time it was ever done in Memphis and certainly the first time ever at St. Jude," Wallace says.

Alex has been migraine-free since that operation.

"I got my life back. I was able to get back to school. I was able to sleep and eat," she says. This fall, she will enroll in college, where she plans to study art, design and architecture.

"St. Jude has been about quality of life," says Alex's mom, Patti. "They don't just say, 'The tumor is smaller, go home.'"

"It's unusual for a cancer hospital to offer services as comprehensive as these."

— Robert Wallace, MD

They say, 'You are not living life the way you need to. How can we make it better? What can we do to have you personally, emotionally and socially recover from the disease?'"

Dazzling possibilities

Plastic surgery services are improving quality of life for other St. Jude patients, as well.

"We do a lot of things that people would not necessarily think about as being plastic surgery," Wallace says.

His team helps children who need reconstruction after limb-salvage procedures. Plastic surgeons are also involved in the treatment of head and neck cancers and in nerve reconstruction.

One service offered by St. Jude plastic surgeons is facial reanimation, which addresses the facial paralysis that can occur after brain tumor treatment.

Other facilities offer operations that may help with a smile, but those procedures do not generally produce a smile that is coordinated with the non-paralyzed portion of the face. Wallace and Konofaos use layered techniques to produce a coordinated smile.

"We're very excited about this program because most of these children have one goal: To have a coordinated smile," Wallace says. "And the only way they can have that is if they have this kind of surgery."

Now, *that's* something to smile about. ■



Robert Wallace, MD (at right) performs a surgical procedure assisted by (from left) Lin Jing, MD, and Brenda Strickland, RN.



Lessons from the

SANDBOX

Play fair. Take naps. Hold hands. Share. By sharing information and resources with colleagues worldwide, St. Jude scientists hope to find cures for pediatric solid tumors.

By Elizabeth Jane Walker

It's one of the first lessons you learned in preschool: the fine art of sharing. By reaching out to others, the fun is multiplied. Now, St. Jude Children's Research Hospital is applying that playground practice to childhood solid tumors—with dramatic results.

St. Jude researchers and clinicians have built the world's most extensive repository of pediatric solid tumor samples and drug screening data. And they are sharing that bounty with scientists around the world.

It's a project with no place for ego or financial gain. The goal is simple: to advance the care of children with solid tumors worldwide.

Hold hands, play together

In 2010, St. Jude and Washington University School of Medicine joined forces to decode the genomes of childhood cancer patients. St. Jude scientists immediately began to ask,

"How will we test drugs against tumors with these specific mutations?"

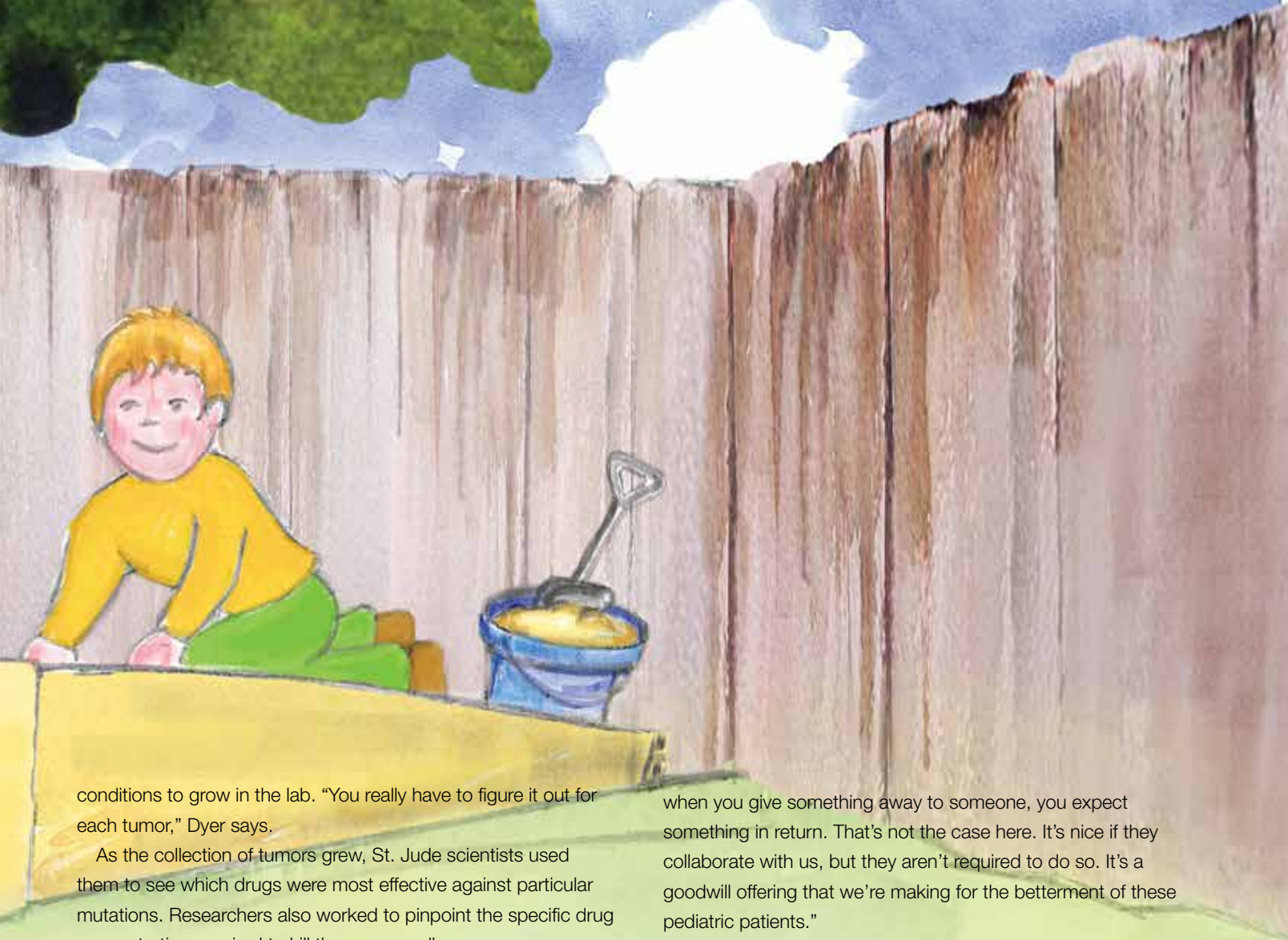
They didn't have many choices. Existing tumor cell lines that had been cultured for decades were not necessarily useful. Then St. Jude scientists realized they might be able to use patient tissue samples to grow tumors in the lab.

"We see a large number of diverse patients here—everything from the most common pediatric solid tumors, like neuroblastoma, to incredibly rare tumors. And that gives us an opportunity to build a resource that spans the continuum of solid tumors. We have the expertise and the infrastructure to grow them all in the laboratory," explains Howard Hughes Medical Institute Investigator Michael Dyer, PhD, of St. Jude Developmental Neurobiology.

Share your toys

In a clinical trial led by St. Jude oncologist Sara Federico, MD, nearly 200 patients undergoing surgery for solid tumors agreed to let scientists use part of their tumor samples for the project. After removing identifying information to protect patient confidentiality, scientists went to work. They sequenced each tumor, examined the anatomy of the tissue and scrutinized the structure using technology called electron microscopy.

Each tumor has its own quirks, requiring its own set of



conditions to grow in the lab. “You really have to figure it out for each tumor,” Dyer says.

As the collection of tumors grew, St. Jude scientists used them to see which drugs were most effective against particular mutations. Researchers also worked to pinpoint the specific drug concentration required to kill the cancer cells.

The amount of data generated is vast.

“As we started growing more and more tumors, we started to realize, ‘Wow! This is a really amazing collection,’” Dyer recalls. “We could sit on this information and be the only ones in the world to have it, but that wouldn’t achieve our goal.

“So we thought: ‘Why not share it?’”

Give freely

The Childhood Solid Tumor Network enables St. Jude to do just that. Researchers around the globe can obtain data and tumor samples free of charge, with no obligation for further collaboration. Requests for resources are reviewed by Dyer and Alberto Pappo, MD, co-leaders of the Developmental Biology and Solid Tumor Program, in consultation with an advisory panel. Requestors are responsible only for shipping costs.

Beth Stewart, MD, of St. Jude Oncology, has shipped samples to colleagues throughout the United States, as well as to Asia, Australia and Europe. She cites several aspects of the program that make it remarkable.

“We have the largest bank of resources available,” she says, “and the way we’ve created these resources is unique. Usually

when you give something away to someone, you expect something in return. That’s not the case here. It’s nice if they collaborate with us, but they aren’t required to do so. It’s a goodwill offering that we’re making for the betterment of these pediatric patients.”

Be thankful

The Childhood Solid Tumor Network is helping St. Jude build robust partnerships around the globe. Many researchers who have obtained data and samples have followed the hospital’s lead, eagerly sharing their results. Not only does the network forge new relationships, but it unites scientists and clinicians in a common goal.

“It’s a really powerful message,” Dyer says, “to be able to say, ‘It’s free. All you do is pay for shipping. We want you to take this and follow your ideas.’ Already several papers have been published using data from this project. It’s pretty amazing to think about how far-reaching the impact will be.”

Sharing data, expertise and resources has its own inherent rewards, Stewart says.

“We have such phenomenal resources and incredible collaborators,” she says. “I feel incredibly blessed every day I walk through the doors of the hospital that I have the opportunity to do this type of work.” ■

More information about the Childhood Solid Tumor Network:

Visit stjude.org/CSTN or email CSTN@stjude.org

JUST THE RIGHT CHORD

This professional musician supports the work of St. Jude through a charitable gift annuity.

David Tweed has traveled the world as a member of the International Flute Orchestra, playing in venues from Santiago, Chile, to St. Petersburg, Russia, for nearly 20 years.

An accomplished musician, he plays a variety of wind instruments including clarinet, saxophone and oboe. “But flute is the one I enjoy the most,” he says. Although he is a classical music enthusiast, Tweed often plays with pit orchestras, accompanying popular musicals, near his home in Florida.

His audiences may be surprised to know the flutist in the orchestra earned a degree in electrical engineering from Massachusetts Institute of Technology. In fact, music is something of an encore career for Tweed, who worked on medical instrumentation and laser systems for many years before turning to entrepreneurship. While living in Boston, he co-founded a company based in Germany that developed laser-beam deflection hardware.

Perhaps not surprisingly, Tweed is accustomed to having to explain his work with lasers.

“There are industrial and some medical applications, such as in genome reading equipment,” he says. “This was also among the earliest commercialized ways of doing 3-D printing, in the early 1990s.”

Tweed began supporting St. Jude Children’s Research Hospital only recently, but his affinity for the cause has roots in his childhood.

“I grew up watching Danny Thomas on TV in the ‘50s,” he

explains. “It stuck with me, and especially what he did when he founded St. Jude.”

Tweed has included a gift for St. Jude in his estate plans, and he established a generous St. Jude charitable gift annuity last year as well.

“It had been in the back of my mind over the years to do something for St. Jude, and the time was right,” he says.

Tweed chose this unique giving vehicle, which provides payments for life at a set rate.

“After I sold my remaining interests in my company, a charitable gift annuity provided a tax deduction, which I needed,” he says. “And the lifelong payments are a benefit for me, too.”

A native New Yorker, Tweed has traded Boston’s snow for Florida’s warmer climate, giving up downhill skiing in favor of sailing.

“I try to get out on my sailboat whenever I can,” he says.

A man of many interests, Tweed used to be a pilot as well—though these days he opts to sit in a passenger seat during his frequent travels.

St. Jude is on Tweed’s travel itinerary this year so he can see firsthand the pioneering work he is helping make possible. For this musician, supporting the mission of St. Jude strikes just the right chord.

Learn more about estate gifts or how a charitable gift annuity may benefit you at stjude.org/legacy. ■



By Elizabeth Jane Walker

James R. Downing, MD, unveils a bold plan for saving the lives of children around the globe.

At first, you might assume the president and chief executive officer of St. Jude Children's Research Hospital is posing a rhetorical question: "If not St. Jude, then who?" he asks.

But the fire in his eyes and the determination in his voice make it clear that James R. Downing, MD, is issuing a battle cry, not presenting an idle query.

Downing is the man with a plan: a \$7 billion, six-year strategy for conquering cancer and other childhood diseases.

"We're in a war against cancer with innocent children dying every day," he says. "As stewards of this extraordinary institution, we must continuously ask: 'Are we doing enough to win that war?'"

A year ago, with that challenge in mind, Downing enlisted the help of 180 individuals from across the institution who were organized into 15 working groups. Together, they created a bold plan for success on three fronts: in the clinic, in the laboratory and around the world.



“WE MUST DO what others cannot do.”

FORWARD MOMENTUM

During the past five decades, St. Jude has made remarkable progress toward curing childhood cancer and other life-threatening diseases. Downing wants to build on that success, pushing cure rates ever higher, while developing tailored treatments to decrease side effects.

To do that, St. Jude plans to double the number of children enrolled in St. Jude-led clinical trials. The hospital will also initiate a proton therapy program, create a national referral clinic for rare pediatric cancers, and launch a program aimed at harnessing the immune system to combat cancer.

The hospital's survivorship efforts will increase as well, with 6,000 childhood cancer survivors returning to campus as part of the St. Jude LIFE program. This program currently involves about 4,500 long-term survivors. St. Jude brings those survivors to campus for clinical testing aimed at increasing the quality and length of life for current and future survivors.

Five years from now, 600 new cancer patients will arrive on campus every year. Each of those children will have their genomes sequenced as part of the hospital's clinical genomics program. St. Jude recently created a new Cancer Predisposition Program for the nearly 10 percent of St. Jude cancer patients who have inherited genetic mutations that may increase their cancer risk. In 2015, the hospital took the next step in that effort by opening its Genomes for Kids protocol.

“These are big efforts that no other single institution can do,” Downing says.

CLINICAL CONNECTIONS

One key initiative is the creation of a St. Jude-funded Clinical Research Consortium, a global consortium of institutions that will develop clinical trials for rare, aggressive diseases. About a dozen of the world's top institutions will be invited to collaborate on developing and running highly complex clinical trials.

“One example is infant leukemia,” Downing explains. “Each institution might see only a couple of cases a year, so nobody makes progress toward curing it. But with

the St. Jude Clinical Research Consortium, we can bring together about a dozen of the top institutions from around the globe to collaborate on protocols. With this coordinated approach, we can make that progress.”

MORE THAN CANCER

In the coming years, children with sickle cell disease, coagulation disorders and bone marrow failure syndromes will arrive on campus in ever-increasing numbers.

Research in those areas will extend beyond symptom management to cures.

“Most programs in sickle cell anemia have focused on decreasing symptoms,” Downing explains. “Well, why not try to cure it? Can we use gene therapy to do that? Can we use gene editing to cure it? Can we find better drugs that would essentially reverse the disease? We're expanding our research efforts in those areas.”

St. Jude will create a long-term follow-up study for patients with sickle cell disease to extend their lifespan and quality of life. That program will be supported by genome sequencing studies for children with sickle cell disease. These studies will identify the genetic variants and determine how they influence patients' outcomes and complications.

For all children and families who come to campus, Downing aims to create the gold standard for patient care.

“Our patients love St. Jude,” he says. “But we want to enhance the patient and family experience to make it even better—from their first contact with the hospital to their arrival, housing and transportation.”

SOLVING SCIENTIFIC MYSTERIES

Even though science has come a long way, gaps still exist in our knowledge. Why do certain mutations lead to cancer and other life-threatening diseases? Discoveries in that area will enable clinicians to design more effective treatments. To answer those kinds of questions, St. Jude will set up collaborative research efforts across the globe—bringing together the quickest minds and most innovative thinkers to work on particular knowledge gaps.

Through the Collaborative Research Program, St. Jude will organize global teams of scientists to address these

ST. JUDE PRIORITIES

crucial scientific questions. Researchers from government agencies, academic institutions and industry will strive toward a common goal.

"These are fundamental laboratory problems," Downing says, "but it requires people with multiple kinds of expertise to work collaboratively to address those knowledge gaps."

A GLOBAL VISION

Downing says St. Jude has the obligation and the ability to help children around the world. Today, more than 80 percent of children with cancer live in low- and middle-income countries. More than half of those will die from their diseases.

The St. Jude International Outreach Program is dedicated to changing that sobering statistic.

"Our goal is to ensure that pediatric cancer patients worldwide have access to care," Downing says.

The International Outreach Program already encompasses 24 partner sites in 17 countries. Those relationships have resulted in astounding success. In Brazil, St. Jude efforts have helped increase the survival rate for children with acute lymphoblastic leukemia from 32 percent in the 1980s to 63 percent today. In El Salvador, that rate has risen from 5 percent in 1993 to 48 percent today.

But survival rates in those countries and others still have a long way to go. During the next five years, St. Jude will enhance its International Outreach Program, saving the lives of countless children around the world.

The hospital's plans are extensive; the potential exciting. But Downing says the aim is simple. It's a plan that hinges on the support of scientists, clinicians, staff and donors.

"Our goal is to accelerate progress," he says.

"How do we use our resources and our position and our knowledge to do that?"

"We're at a point in our hospital's history where it's clear we can do more. Because of our success, we have the opportunity and the responsibility to establish an agenda that will accelerate progress toward advancing cures for pediatric catastrophic diseases.

"We must do what others cannot do." ■

James R. Downing, MD
President and Chief Executive Officer
with St. Jude patient Juliana Judge

Clinical Care

- Increase the number of patients treated on St. Jude-led clinical trials
- Set the standard for pediatric cancer care delivery
- Advance clinical care programs for children with non-malignant blood diseases

Research

- Strengthen basic lab and clinical research programs
- Continue to create and run high-complexity clinical trials
- Establish the benchmark for precision medicine
- Determine the optimal use of proton therapy for brain tumor, solid tumor and Hodgkin lymphoma
- Develop a world-class program to harness the immune system to treat childhood cancers

Global

- Expand the International Outreach Program
- Develop a St. Jude-funded global Clinical Research Consortium
- Organize global teams of scientists to collaboratively address high-priority scientific questions



PETER BARTA



PETER BARTA

Michelle Churchman, PhD, and Charles Mullighan, MD, MBBS

Two leukemias. One shared genetic alteration. Why are the outcomes so different?

The mystery has been solved about why two types of leukemia that share the same genetic alteration behave so differently and often have quite different outcomes. The discovery—made by St. Jude researchers and published in the journal *Cancer Cell*—offers a promising new cancer treatment strategy.

BCR-ABL1 is a well-known genetic alteration associated with several different types of leukemia. The alteration is often present in both chronic myeloid leukemia (CML) and a subtype of acute lymphoblastic leukemia (ALL) called *IKZF1*-mutated *BCR-ABL1* ALL. Yet, these cancers behave much differently. CML, a slow-growing cancer that occurs mostly in adults, is often sensitive to targeted therapies called tyrosine kinase inhibitors, or TKIs. These drugs are a precision medicine success story. TKIs have transformed the outlook for patients with CML by targeting the abnormal *BCR-ABL1* alteration.

In contrast, *IKZF1*-mutated *BCR-ABL1* ALL is an aggressive disease that occurs in children and adults. TKIs are less effective against this high-risk ALL subtype, and patients are less likely to be cured.

Scientists have discovered how mutations in a gene called *IKZF1* or *IKAROS* drive the development of ALL rather than CML, resulting in an aggressive leukemia that is less responsive to TKIs. In this study, researchers showed that *IKZF1* mutations cause certain white blood cells with *BCR-ABL1* to retain stem-cell like characteristics. That leads to a more aggressive disease and may help leukemic cells hide from targeted therapies.

“The research shows why, in this era of targeted therapies, patients with *IKZF1*-mutated *BCR-ABL1* ALL fare so poorly,” said Charles Mullighan, MD, MBBS, Pathology. “That insight led us to a promising new treatment strategy that we are pursuing in the laboratory.”

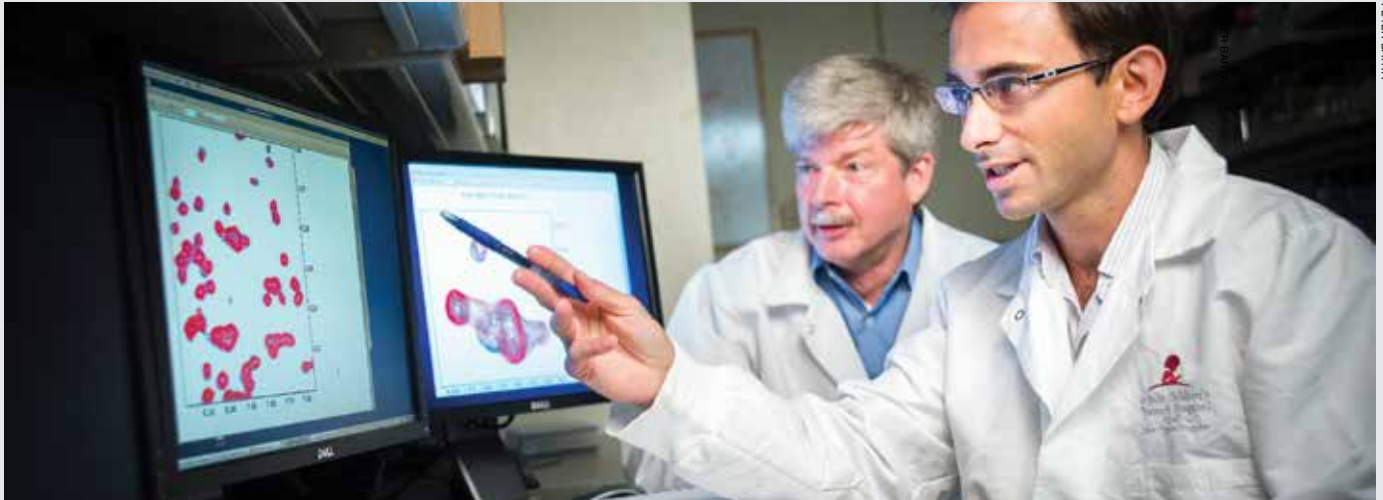
Eighteen genes identified that contribute to brain tumor

St. Jude scientists have identified eight tumor-promoting oncogenes and 10 tumor suppressor genes that converge on a handful of cell functions and help to launch the pediatric brain tumor ependymoma.

The findings dramatically expand understanding of ependymoma, which until now has been linked to alterations in just two genes. The tumor is discovered in 150 to 200 children and adults annually in the U.S., where it is the third most common pediatric brain tumor.

Researchers expect insight from this study will lead to new chemotherapy agents and more effective treatment of a cancer that remains incurable in about 28 percent of pediatric patients.

The study appeared in the scientific journal *Nature Genetics*.



Richard Kriwacki, PhD (at left), and Ariele Follis, PhD

Discovery yields promising strategy for making tumor cells easier to kill

St. Jude scientists have discovered another way the tumor suppressor protein p53 earns its title as guardian of the genome.

p53 is one of the most famous cancer-related molecules. Its job is to keep tumors from forming. The protein does that by prompting stressed or damaged cells to die or stop dividing. In most cancers, the protein or the pathway that controls p53 does not work properly.

p53 is best known for working in the cell nucleus. But earlier research showed p53 also works outside the nucleus of cells to trigger cell death.

Scientists have now discovered how p53 achieves the latter task. The results also suggest how small molecules may be used in the future to trigger the same mechanism to help kill cancer cells.

“These results expand our understanding of how p53 regulates cell behavior and highlight a possible new way to make tumor cells easier to kill,” said Richard Kriwacki, PhD, of St. Jude Structural Biology.

The study appeared in the journal *Molecular Cell*.

Scientists uncover how a common mutation causes brain diseases

Researchers have discovered how the most common genetic cause of two devastating brain disorders disrupts the normal function of nerve cells. The finding suggests a possible new treatment strategy for amyotrophic lateral sclerosis (ALS), which is also called Lou Gehrig disease, and frontotemporal dementia (FTD).

The mutation occurs in a gene named *C9ORF72*.

Scientists at St. Jude and the University of Massachusetts Medical School found the mutation blocks movement of RNA and other molecules in and out of the cell’s command center or nucleus. That disrupts important cell processes, such as assembling the proteins that do the work of cells.

Investigators checked nerve cells generated from people with the mutation and found that RNA built up in the cell nucleus.

That did not happen in nerve cells from people without the mutation. RNA also did not build up in the nucleus of non-nerve cells from patients with the mutation.

ALS and FTD involve the deterioration and death of nerve cells in the brain and spinal cord. That leads to muscle weakness, paralysis plus other symptoms as well as problems walking or swallowing. There are no treatments to halt or reverse the process. Most patients die within five years of diagnosis.

“This study reveals the key defect that we need to reverse in treatment, possibly by knocking out or silencing the mutant gene,” said J. Paul Taylor, MD, PhD, Cell and Molecular Biology chair and a Howard Hughes Medical Institute investigator.

The study appeared in the journal *Nature*.



Monika Metzger, MD (at left), and Elisabeth Adderson, MD

Improving diagnosis of chest tumors

Is it cancer or a fungal infection? For children with a mass or lump in their chest cavity, the answer is not always immediately clear.

No single sign or test can reveal which masses are cancerous and which are benign tumors caused by the fungal infection histoplasmosis. For many patients, a diagnosis requires biopsy surgery.

Research led by St. Jude investigators could lead to a faster and simpler approach.

Scientists found two previously overlooked clues in the health records of 131 children and teens with chest masses. Patients with enlarged lymph nodes in the neck and low levels of certain white blood cells were more likely to have cancer. Masses located in front of the heart were also more likely to be malignant. The results were published in the *Journal of Pediatrics*.

Researchers now plan to track whether factors discovered in this study will allow faster and more accurate diagnoses without surgery.

“The problem has been that there are both benign and malignant causes of these masses,” said Elisabeth Adderson, MD, of St. Jude Infectious Diseases. “In some parts of the country, particularly in the Ohio and Mississippi River valleys, the masses are more likely to be caused by infection than cancer.”

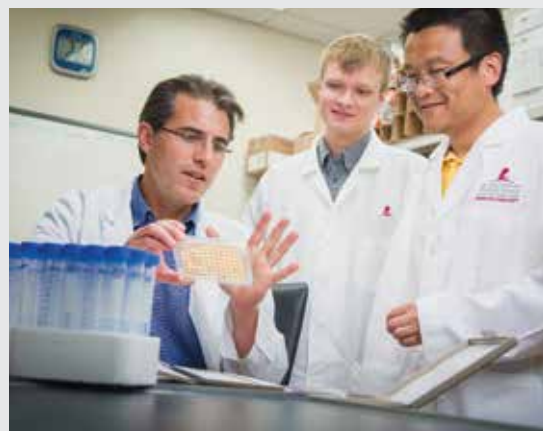
Teaching old cells new tricks

Hopes for teaching “old” cells new tricks got a boost from research led by St. Jude scientists.

They have developed a new way to make replacement eye cells from adult nerve cells. The process involves coaxing mature cells to return to an immature state. These are the induced pluripotent stem cells that can then be reprogrammed to become different adult cells. One promising use of the cells is to restore vision lost to age-related macular degeneration, retinitis pigmentosa and Stargardt’s disease.

The scientists also found a way to compare the ability of different types of stem cells to create specialized eye cells called retinal cells. Investigators learned that stem cells from certain eye cells produce more vision replacement cells than do certain skin stem cells.

“This research helps to answer an important scientific question by showing that the source of the stem cell makes a difference,” said Michael Dyer, PhD, of St. Jude Developmental Neurobiology and a Howard Hughes Medical Institute investigator. The research was published in the journal *Cell Stem Cell*.



(From left) Michael Dyer, PhD; Dan Hiler, PhD; and Xiang Chen, PhD

LONG LIVES, WELL LIVED

By Kathryn Sharkey



Brennan Bergeron
Cancer Survivor

Recent research shows changes in childhood cancer treatment have extended the lives of childhood cancer survivors diagnosed within the past three decades.

As he takes a break from shadowing physicians at St. Jude Children's Research Hospital, 22-year-old Brennan Bergeron swears he never intended to pursue a career in medicine.

"But no matter how much I tried to stay away from it, I ended up finding my way back," admits the former St. Jude patient.

The pre-medical student and long-term survivor of acute lymphoblastic leukemia (ALL) recently returned to the hospital to shadow staff who played a part in his cancer treatment, as well as Greg Armstrong, MD, of St. Jude Epidemiology and Cancer Control.

Armstrong is principal investigator of the Childhood Cancer Survivor Study (CCSS), the world's largest comprehensive resource for survivorship research. A collaborative, multi-institutional study, the CCSS focuses on identifying and reducing the long-term effects of cancer treatments for survivors.

As any cancer survivor can attest, health concerns don't stop once the cancer is gone. Armstrong is one of many St. Jude physician-scientists working to identify the long-term effects of cancer and its treatment, and to prevent those effects in future survivors.



Greg Armstrong, MD
Epidemiology and
Cancer Control

Looking long term

The CCSS follows more than 35,000 childhood cancer survivors whose cancers were diagnosed and treated between 1970 and 1999, when they were ages 20 and younger.

“To be eligible, these children had to be five-year survivors of their primary disease,” Armstrong explains.

At its 2015 annual meeting, the American Society of Clinical Oncology (ASCO) highlighted results of a CCSS study. Armstrong was lead author of the study, which ASCO identified as one of four being presented that had the greatest potential to affect patient care.

The study reported that the 15-year death rate among five-year survivors is lower for survivors diagnosed in more recent eras, a decrease that coincides with changes in pediatric cancer therapy and follow-up care. Those changes included reductions in the use and dose of radiation therapy and chemotherapy drugs called anthracyclines. These therapies were used to treat such cancers as Hodgkin lymphoma; Wilms tumor, a cancer of the kidneys; and ALL, the cancer Bergeron had.

Vigilance after treatment ends

Results of the CCSS suggest that doctors have learned how and when to back off of, or reduce, certain therapies and are better about recognizing and managing the long-term effects of treatment. But, certain cancer therapies still leave some survivors with an increased risk of health problems compared with siblings. The possible problems include a higher risk of breast cancer or brain tumors due to radiation, heart disease from chemotherapy or other serious health problems.

These problems may not appear until 15 to 20 years down the road, and the risks increase with time.

“As a clinician, when you walk into the room with a five-year survivor, you celebrate. You throw the confetti. In your mind, you imagine they’ve beaten it—they’re done, and they’re going on about their lives,” Armstrong says. “But the truth is that 18 percent of five-year survivors will be deceased by 30 years from diagnosis—that’s one in five. So, there’s still a battle beyond that five-year point, and our current findings suggest we are improving the rates of long-term survival.”

Soaring survival stats

Although survivors still have a battle to face, it is a good sign that the research is evolving from five-year survivorship to decades-long survivorship. In the 1960s, less than 30 to 40 percent of children survived their cancer, Armstrong says, but currently more than 83 percent of children become five-year survivors.

"It was estimated in 2013 there were over 420,000 survivors in the U.S.," Armstrong says, "and that by 2020 there will be over half a million."

With this growing number of survivors, the focus of doctors and researchers is shifting to how to improve the health-related quality of life for long-term survivors, such as Brennan, into adulthood.

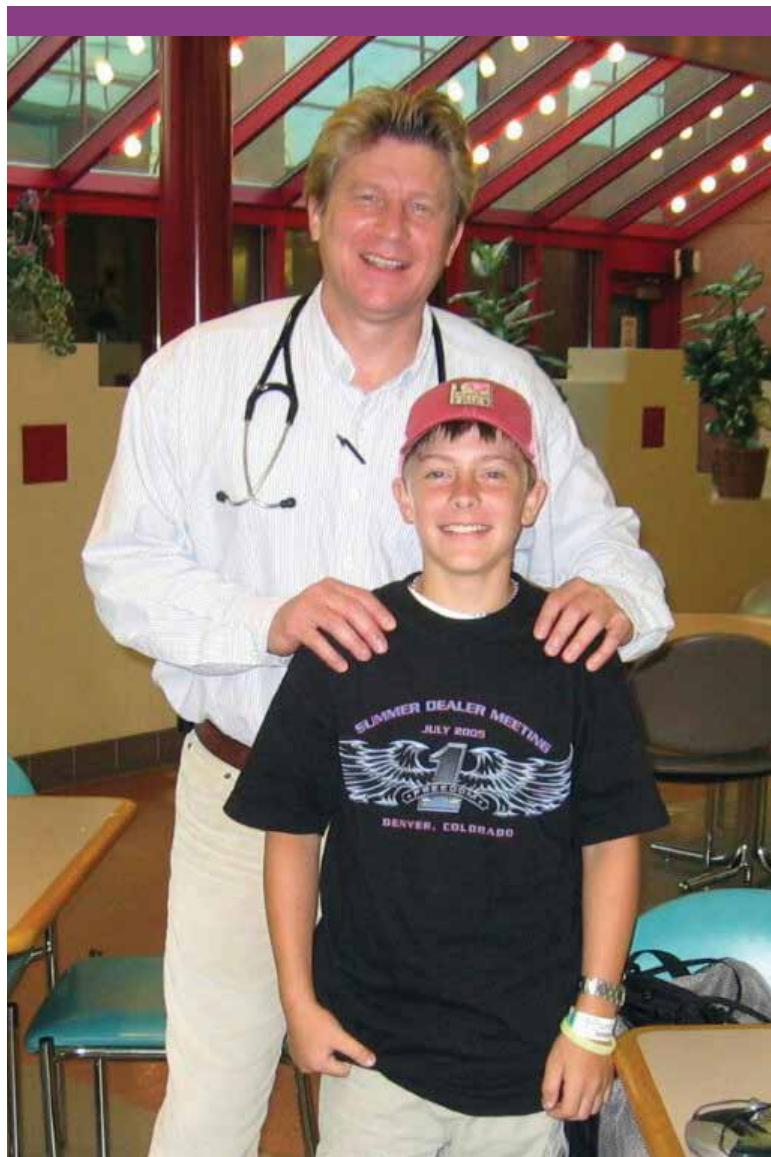
St. Jude for LIFE

One way St. Jude is doing that is through a unique research study called St. Jude LIFE. This initiative is inviting thousands of St. Jude cancer survivors to return to the site of their original treatment for periodic follow-up throughout adulthood. St. Jude is the world's first institution to commit to this type of long-term follow-up research. Discoveries emerging from St. Jude LIFE are helping researchers design new therapies to help future generations of patients and to further improve the quality of life of today's survivors.

Brennan Bergeron's St. Jude LIFE visit also provided an unexpected version of long-term survivor support. His childhood oncologist-turned-mentor, John "Torrey" Sandlund, MD, told him about a St. Jude shadowing program and helped arrange his participation.

Bergeron says his career goal is to follow in Sandlund's footsteps and become a pediatric oncologist at St. Jude. Someday, Bergeron hopes to approach patients and their families and help them feel the sense of security he and his family felt at St. Jude.

"You're in good hands; I'm here to help," he will tell them. "I've been in your shoes before. I'm a former patient, and I know what's possible at this hospital." ■



St. Jude—and John "Torrey" Sandlund, MD—have been part of Brennan Bergeron's life for as long as he can remember. Today, Bergeron is 22 years old and plans to follow in the footsteps of his oncologist-turned-mentor.



By John Juettner

Pay it Forward

Vickie Schutze and her children
(from left) Madison, Gavon Joe and Dalton.

INDIVIDUALS AND ORGANIZATIONS SHOW THEIR SUPPORT
THROUGH ST. JUDE *THANKS AND GIVING*.

Vickie Schutze views her support of St. Jude Children's Research Hospital as coming full circle. Patient stories she heard during a 1999 radiothon inspired her to become a monthly St. Jude Partner In Hope.

"Giving to a charity wasn't something I felt I had budgeted for, but it was certainly something I needed to do," Schutze recalls.

She is now senior manager of executive customer relations for Kmart, the leading corporate partner in the St. Jude *Thanks and Giving* campaign. Schutze actively supports the company's efforts to raise funds and awareness for St. Jude. She has headed individual and team fundraising efforts, from collecting pop tops to encouraging coworkers to make donations in lieu of buying office Christmas gifts. Schutze and her team have also supported special patient activities at the hospital.

The patient stories she heard 16 years ago "touched me so much that I felt I needed to pay it forward," Schutze says. Then the hospital saved the life of someone she loves.

That "someone" is Schutze's nephew, Armand, who received treatment for acute lymphoblastic leukemia at St. Jude.

A holiday tradition like no other

The St. Jude *Thanks and Giving* campaign brings together celebrities, media, retail and corporate partners each holiday season to raise funds and awareness for St. Jude.

Marlo, Terre and Tony Thomas, the children of St. Jude founder Danny Thomas, created the campaign, which has raised more than \$588 million in its 11-year history. The campaign has raised more than \$100 million each of the last two years, thanks to the generous spirit of holiday shoppers, the efforts of individuals like Schutze, and the dedication of partners like Kmart.

"The St. Jude *Thanks and Giving* campaign has become a shining example of the humanity and goodwill that exists throughout the holiday season," Marlo Thomas says. "We are blessed to receive incredible love and support from our corporate partners, their employees and customers. The funds they raise help us stay true to my father's promise that no family pays St. Jude for treatment, travel, housing or food. That's because we believe all a family should worry about is helping their child live."

Last year Kmart raised more than \$17.5 million through the campaign, bringing the company's total contribution to more than \$76.7 million.

"Kmart's long history of success in supporting charitable causes is due to the generosity of our Shop Your Way members, customers and associates," says Alasdair James, Kmart president and chief member officer. "We're proud of the impact our results have made for the families of St. Jude and thankful to St. Jude for its tireless work to defeat cancer and other diseases in children. St. Jude made holiday shopping at Kmart more rewarding, and we're excited to support an organization that clearly means so much to our members."

Kmart's fundraising for St. Jude is part of the company's culture.

"It's not a one-day or one-time push," Schutze says. "It's something that is always thought about and looked at here. As a company we take our partnership and our support of St. Jude very seriously."

In addition to Kmart, more than 70 companies are partners in St. Jude *Thanks and Giving*, including Best Buy, Ann Taylor, Kay Jewelers, Domino's, Williams-Sonoma, Inc., HomeGoods, Mazda, New York & Company, Brooks Brothers, GNC, Dollar General, Claire's, Chili's Grill & Bar, and many more.

Raising awareness for St. Jude

Each year, the St. Jude *Thanks and Giving* campaign brings the work of St. Jude to people across the country when Marlo Thomas and St. Jude patients appear on NBC's *TODAY* show throughout Thanksgiving week.

In addition, special media spots featuring St. Jude celebrity friends and patients air on TV and in movie theaters nationwide, including Regal Entertainment Group, AMC Theatres, Cinemark and Carmike Cinemas. This year, Jimmy Kimmel joins a roster of celebrity supporters that includes Jennifer Aniston, Michael Strahan, Sofia Vergara and Latin GRAMMY winner Luis Fonsi.

Schutze does her part to share the St. Jude story. Each year, she gives gifts featuring the St. Jude logo to spread the word about the hospital's mission.

In 2012, Armand celebrated the end of his treatment at a No Mo' Chemo party. He is now pursuing a career in computer and networking repair. Since attending that party, Schutze has encouraged people to take the time to visit St. Jude, which she calls a magical place.

"It's not just about sick kids there ... they don't treat them like kids with cancer," Schutze said. "They treat them like kids." ■

Marlo Thomas celebrates the St. Jude *Thanks and Giving* season with (from left) Cooper Abbott, Gayatri Linga and Tamara Ford.



JOHN ZACHIER



BY LORI DE MILTO

Last summer, 6-year-old Brett Sutherland learned how to swim without a life jacket and jump off the diving board—proud moments for the little swimmer from Missouri. The most gratifying part for his parents, however, is that he could acquire these skills in his hometown while undergoing treatment for acute lymphoblastic leukemia.

Brett is a patient of St. Jude Children's Research Hospital, which is a four-hour drive from home. But through the St. Jude Affiliate Program, he was able to receive most of his care just minutes away, at Mercy Children's Hospital in Springfield, Missouri.

As a result, the little boy who likes dancing to Michael Jackson songs spent the summer sleeping in his own bed, playing his first season of little league baseball, and spending time with his parents and his older sister, Kately. Mercy Children's Hospital is one of seven affiliate clinics where children can get most, sometimes all, of their St. Jude care closer to their homes.

ST. JUDE AFFILIATE CLINICS MAKE IT EASY FOR FAMILIES TO GET CUTTING-EDGE CARE AND SUPPORT.

PETER BARTA



Carolyn Russo, MD
St. Jude Affiliate Program
medical director

Keeping families together

"Through our affiliate program, children with cancer or blood disorders can receive the St. Jude cutting-edge care, including clinical trials that have led to increased cure rates, in a way that's convenient for the family," says Carolyn Russo, MD, medical director of the program, which includes locations throughout the Southeast and Midwest.

The newest addition to the program is the clinic at Novant Health Hemby Children's Hospital in Charlotte, North Carolina. The clinic opened in April 2015. "Families dealing with children with cancer have incredible stress," says Randy Hock, MD, PhD, director of the affiliate. "Keeping them closer to home keeps families intact and prevents job loss."

Brett's mom, Laura, knows how much easier it is for Brett to get care in their hometown, because she spent more than six weeks in Memphis with him when he was diagnosed. Her husband, Ryan, took off from work for the first week. After that, he and Kately drove to Memphis every weekend.

"Being at home has kept us closer to our support system and has given us a way to connect locally with families who are traveling the same journey we are," Laura says.

Finding cures faster

The affiliate clinics enable St. Jude to share new treatments and support programs with more children and families.

"Increasing the number of affiliates gives us the opportunity to study newer forms of therapy more broadly and, at the same time, in a much more rapid manner," says Larry Kun, MD, clinical director and executive vice president at St. Jude.

Through the affiliates, more children can take part in clinical trials, so doctors and scientists can develop new treatments more quickly.

Some children go to St. Jude in Memphis for clinical trials and then return to the affiliate for most of their care. Doctors at the affiliate clinics work with St. Jude doctors to fashion the best treatment for each child.



Brett Sutherland received much of his St. Jude treatment in his hometown, thanks to the hospital's Affiliate Program.

Children can get most, sometimes all, of their care closer to home through seven St. Jude affiliate clinics.

Expanding care to more regions

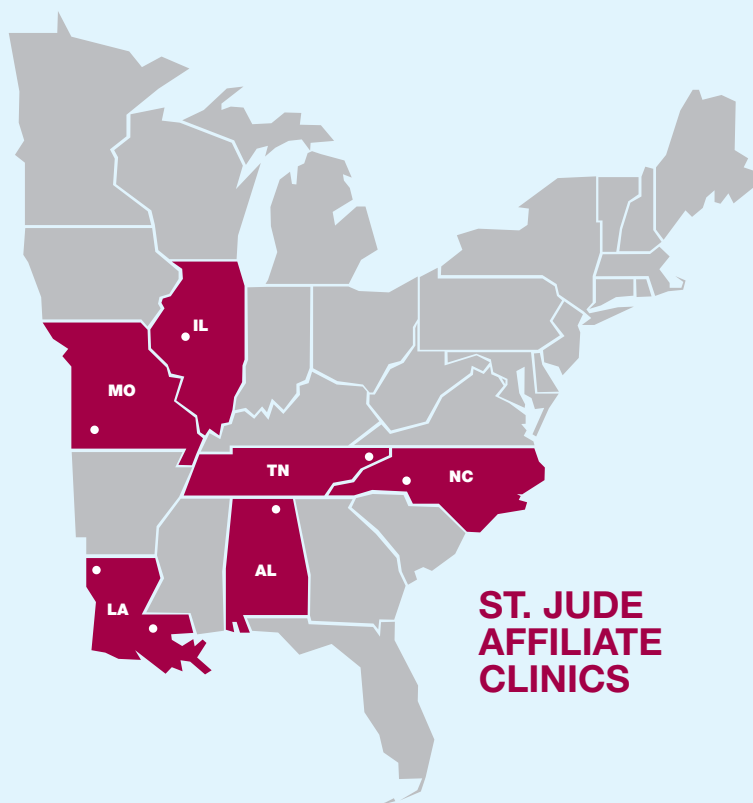
Russo and her team also support the affiliate clinics behind the scenes. They offer training and opportunities for the clinic staff to learn from one another at an annual meeting and through teleconferences and videoconferences.

"Learning is not only from Memphis to the affiliates; it's also from affiliate to affiliate," Russo says. Novant Health, for example, shared its progress in using their unique patient portal with the other affiliates.

About 250 new St. Jude patients visit the affiliate clinics each year, with the number expected to grow. In 2016, St. Jude plans to add an eighth affiliate clinic.

Laura says her family has built close relationships with their affiliate doctor and nurses.

"They feel like family now, and Brett looks forward to seeing them and his other St. Jude friends each week," she says. "Our family is forever grateful for all St. Jude has done for us." ■



The Graduation Gift

Commencement and an exciting future:
Brought to you by St. Jude.

In 2007, life forever changed when my daughter, Laura Adcock, was diagnosed with a brain tumor called medulloblastoma.

Laura was 19 years old at the time. For at least a year, there had been subtle changes in her personality. Then she developed headaches that came and went. With a history of migraines in the family, I didn't give it much thought and just tried to help her cope. When Laura began to vomit in the mornings, doctors diagnosed a digestive disorder. Once, when she was talking to me, Laura had a blank expression on her face as if she were unconscious, yet still sitting up.

After witnessing those symptoms, we returned to the doctor. An MRI revealed two tumors: a large one at the base of her brain and one on top of her brain. The larger one needed to be removed immediately.

Not long after Laura's surgery, I heard from a friend about her experiences at St. Jude Children's Research Hospital.

Scared and not sure of which direction to go, I sent an email to Dr. Amar Gajjar [director of St. Jude Neuro-Oncology and co-leader of the Neurobiology and Brain Tumor Program]. I included only a few sentences, introducing myself and my daughter's condition, and the word "help." He called me on a Saturday night, and we were on a plane by Tuesday.

Class of
2015



With Laura's age and the diagnosis of a childhood tumor, we were thankful that St. Jude was able to treat her. She received chemotherapy and radiation. They organized all of the appointments. They supported us by providing housing, food and travel expenses. Her doctors and nurses were attentive and encouraging. Their attention and care allowed us to focus on fighting this disease. They celebrated her birthdays, honoring her treatment completion with confetti and a party.

We do not know where Laura would be without St. Jude.

Laura has since finished treatment. In May of 2015, she graduated from George Mason University with a BA in English with honors. It is hard to believe that not long ago we were asking for help. That help allowed us to endure some of the toughest times.

Laura is still monitored and checked every year. It is comforting to know that she is still on the St. Jude radar.

St. Jude is a place you hope you never need, but if you do you are thankful that Danny Thomas had a vision, and that his vision lives on. ■

PLEASE **GIVE**
to help her live.



**St. Jude Children's
Research Hospital**

Finding cures. Saving children.

When Gabby was still an infant, her mom noticed a lump growing in her abdomen, which was diagnosed as kidney cancer. After treatment at St. Jude, 2-year-old Gabby is back home, and her cancer is in remission. Because you give, families never receive a bill from St. Jude for treatment, travel, housing or food. Because all a family should worry about is helping their child live.

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Magnificent Magnet

Applause and cheers follow the news that St. Jude has been granted Magnet® designation by the American Nurses Credentialing Center. Magnet status is the highest honor bestowed upon nursing services. Only 7 percent of all U.S. hospitals have achieved this recognition. The Magnet Recognition Program® serves as a benchmark for patients to measure the quality of nursing care they can expect to receive at a hospital.

PETER BARTA



Finding cures. Saving children.