

# Promise

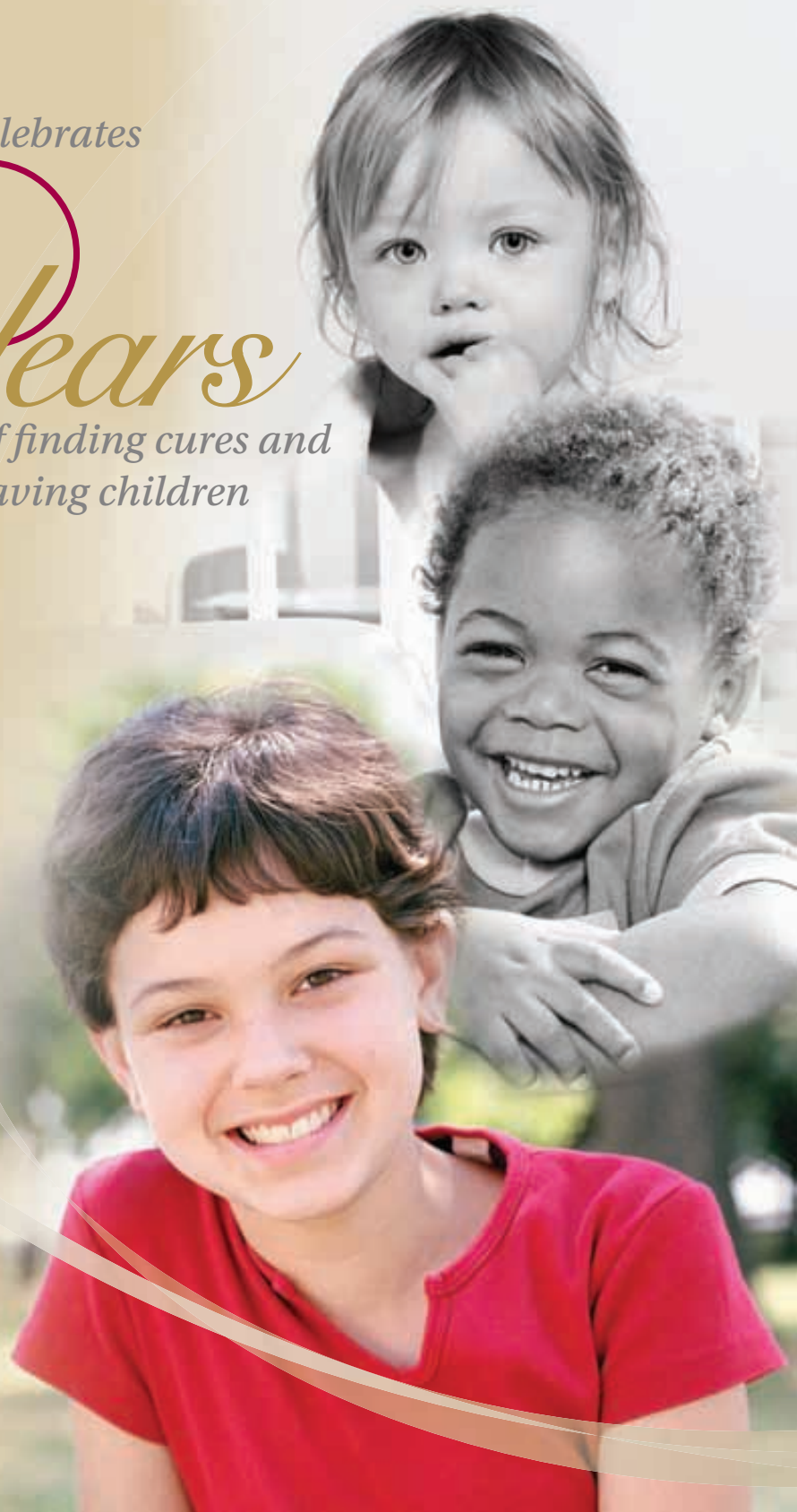
SPRING 2012



*St. Jude celebrates*

**50**  
*Years*

*of finding cures and  
saving children*



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Track the astounding progress that has occurred at St. Jude during the past five decades.

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From Dream to Reality

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St. Jude Children's Research Hospital's mission is to advance cures, and means of prevention, for pediatric catastrophic diseases through research and treatment. Consistent with the vision of our founder, Danny Thomas, no child is denied treatment based on race, religion or a family's ability to pay.

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## Promise

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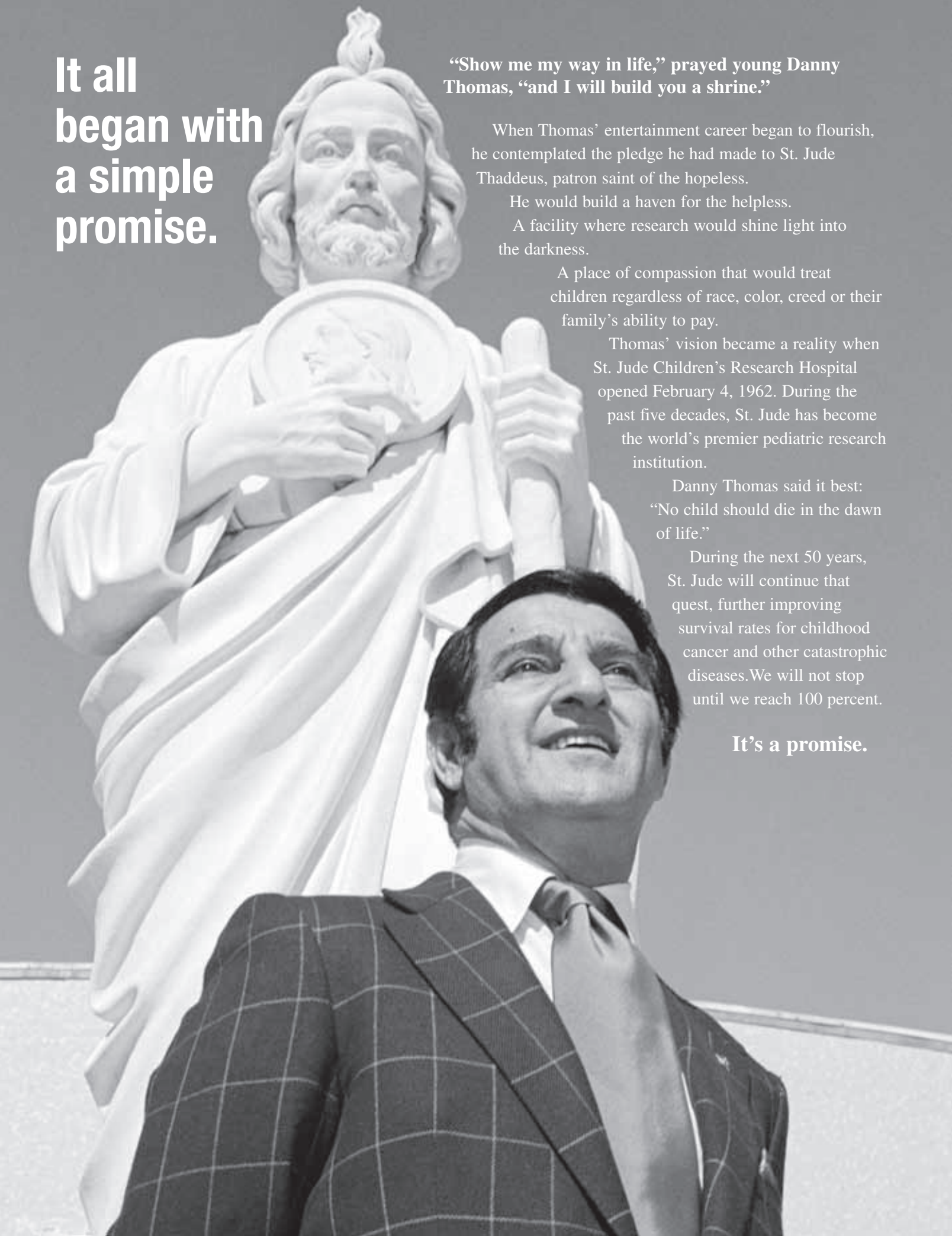
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**It all  
began with  
a simple  
promise.**

**“Show me my way in life,” prayed young Danny Thomas, “and I will build you a shrine.”**

When Thomas’ entertainment career began to flourish, he contemplated the pledge he had made to St. Jude Thaddeus, patron saint of the hopeless.

He would build a haven for the helpless.

A facility where research would shine light into the darkness.

A place of compassion that would treat children regardless of race, color, creed or their family’s ability to pay.

Thomas’ vision became a reality when St. Jude Children’s Research Hospital opened February 4, 1962. During the past five decades, St. Jude has become the world’s premier pediatric research institution.

Danny Thomas said it best: “No child should die in the dawn of life.”

During the next 50 years, St. Jude will continue that quest, further improving survival rates for childhood cancer and other catastrophic diseases. We will not stop until we reach 100 percent.

**It’s a promise.**



# 1960s

*"It took a rabble-rousing, hook-nosed comedian to get your attention, but it took your hearts and your loving minds and your generous souls to make this fabulous dream come true.... If I were to die this minute, I would know why I was born."*

*– Danny Thomas at St. Jude opening, February 4, 1962*

## ***"Your child has cancer."***

No words strike more fear into a parent's heart. In the early 1960s, those words meant almost certain death. The overall survival rate for childhood cancer was less than 20 percent; for the most common form of childhood cancer, acute lymphoblastic leukemia (ALL), it was 4 percent. In spite of those appalling statistics, no institution was dedicated to fighting the scourge of childhood cancer, sickle cell disease and other catastrophic disorders. Then, St. Jude opened. The battle against catastrophic childhood diseases had begun.

1958



his work at the University of Tennessee at Memphis—subsequently publishes the first comprehensive study of the disease and its impact on the African-American population.

◀ Even before the hospital is built, research begins. ALSAC, the fundraising organization for St. Jude, presents a \$10,000 Plough Inc. grant to Lemuel Diggs, MD, for his work on sickle cell disease. Diggs—who conducts

1962

**February 4, 1962** — The hospital opens in Memphis, Tennessee, before a crowd of 9,000 people. ▼



1965

The first immunologic method to diagnose solid tumors in children is developed at St. Jude.

1966

A group of St. Jude patients are the first ALL patients to ever be successfully taken off therapy, based on evidence that remission can be sustained.

1968

St. Jude researchers find that chemotherapy is effective against Ewing sarcoma, one of the most frequent malignant bone tumors in children. When combined with radiation, this treatment causes the survival rate to improve significantly.



*In 1966, Pat Patchell (shown holding his boyhood portrait) was one of the first patients ever successfully taken off therapy for acute lymphoblastic leukemia, based on evidence that remission could be sustained. “How can you express enough thanks, when you were in dire straits and they saved you? It really is the greatest place,” he says, 47 years later.*



◀ St. Jude hires a scientific maverick to lead the fledgling hospital. Unlike most of his peers, Donald Pinkel, MD, has the audacity to believe that cures are possible. His unconventional approach toward the treatment of ALL initially draws objections from many in the scientific community. But thanks to a revolutionary “Total Therapy” regimen combining multiple anticancer drugs with radiation treatment, the survival rates begin to inch upward. Each of the Total Therapy trials builds upon the success of preceding studies—an approach that continues to this day.



◀ St. Jude opens during a turbulent era in American history. The star-shaped building designed by renowned African-American architect Paul Revere Williams immediately becomes the region’s first fully integrated hospital. The integration of St. Jude also extends to the Memphis hotel industry. In order to house St. Jude families, a facility must agree to offer housing to anyone, regardless of race.

By the end of the hospital’s first year of operation, more than 30 research projects have been instituted and four have been completed.

## 1969

“This is the hospital for catastrophic diseases, and the most catastrophic problem in children is malnutrition,” observes Pinkel in the hospital’s 1969 annual report. St. Jude research had shown that 25 percent of low-income children in Memphis were anemic, about one-third had parasitic infections and about 10 percent had growth impairments. St. Jude enrolls thousands of local infants in a successful nutritional program, which serves as the prototype for WIC, the federal health and nutrition program for women, infants and children.

# 1970s

*"If you really want to get the most out of your money, invest in research. If someone discovers a new drug, it can be used for decades—even centuries, maybe—and affect millions of kids. That's why St. Jude is a research institution. Although the research is done in Memphis, it applies to children all over the world."*

*—Walter Hughes, MD, former Infectious Diseases chair*

## 1970

St. Jude issues a statement that would have been impossible a decade before: "Leukemia can no longer be considered an incurable disease."

## 1971

Researchers discover "calmodulin," a small protein that regulates many key activities within living cells.

## 1972

The hospital publishes a study that shows a 50 percent survival rate for acute lymphoblastic leukemia (ALL) using a combination of chemotherapy and radiation. This achievement revolutionizes leukemia therapy worldwide.

The St. Jude Midwest Affiliate Clinic opens in Peoria, Illinois—the first of six domestic affiliate clinics nationwide.

Hospital Director Donald Pinkel, MD, receives the Albert Lasker Clinical Medical Research Award for his contribution to the development of combination therapy for cancer. ▼



## 1973



▲ Alvin Mauer, MD, becomes the hospital's second director.

## 1975



◀ St. Jude becomes the first hospital to identify important subtypes of ALL, including T-cell leukemia. This finding, which proves that ALL is not a single disease, leads to better risk classifications, new research directions and improved treatment.

A new drug combination is found to be effective against leukemia that recurs after initial treatment. This leads to improved therapy for hundreds of leukemia patients, especially those with disease that is at high risk for early failure.

The ALSAC Tower opens to house expanded St. Jude research programs. At its opening, then-President Gerald Ford calls it "a great day for our nation and for the children of our country stricken with catastrophic diseases." In 2005, the building is renamed in honor of Richard C. Shadyac Sr., ALSAC's fourth CEO.▶





*Angel Crum recently traveled from Ohio to Memphis to participate in the St. Jude LIFE long-term follow-up study. She marveled at the changes that have occurred since she underwent treatment for embryonal carcinoma in the 1970s and 1980s. “As soon as the plane touched down, I had all these thoughts going through my head,” she muses. “What Danny Thomas did—not only for me but for other kids—is a blessing. The doctors said that I wouldn’t live past the age of 12, but here I am! My motto is, “I’m on an incredible journey through life.”*



1976



◀ The World Health Organization (WHO) designates St. Jude as a Collaborating Center for the study of the transmission of influenza from animals to humans.

1977

Clinicians develop a treatment that is effective for 55 percent of patients with neuroblastoma, the second most common solid tumor in children.



▲ The hospital launches the first major effort to understand the lifelong progression of sickle cell disease.



◀ St. Jude develops a treatment that not only cures a type of pneumonia frequently fatal to children with compromised immune systems, but also prevents that disease as well as other bacterial infections. The treatment becomes even more important when it is shown to prevent the same type of pneumonia in patients with AIDS.

# 1980s

*"There are two kinds of people in this world: The givers and the takers. The takers sometimes eat better, but the givers always sleep better."*

– Danny Thomas

1983



◀ Joseph Simone, MD, becomes the hospital's third director.

Researchers discover that children with acute lymphoblastic leukemia (ALL) can differ by as much as 10-fold in their ability to clear antileukemia drugs from their blood.

1985

The hospital's new brain tumor program begins accepting patients. Unlike other hospitals, St. Jude provides an integrated and personalized treatment plan for each child. ▼

1984



A St. Jude patient with sickle cell disease is the first to be cured with a bone marrow transplant.

Researchers pinpoint the first two specific genetic translocations known to cause ALL.

Scientists find evidence that some patients have a mixture of two acute leukemias: myeloid and lymphoid.



◀ Scientists develop a novel method to identify patients with neuroblastoma who are likely to have a poor response to therapy. This information allows clinicians to concentrate on this high-risk group while sparing others the toxicity of intensive treatment.

Rearrangement of the genetic material within human chromosomes is found to be an important factor in how a child with leukemia responds to treatment.

St. Jude establishes a clinic specifically for cancer survivors. Today, the After Completion of Therapy Clinic is the world's largest long-term follow-up clinic for pediatric cancer patients.

▲ Investigators discover that childhood leukemia patients who can retain anti-cancer drugs longer in higher concentrations are more likely to become long-term survivors than patients whose bodies remove the drug more rapidly. This marks the beginning of individualizing drug treatments for each child.

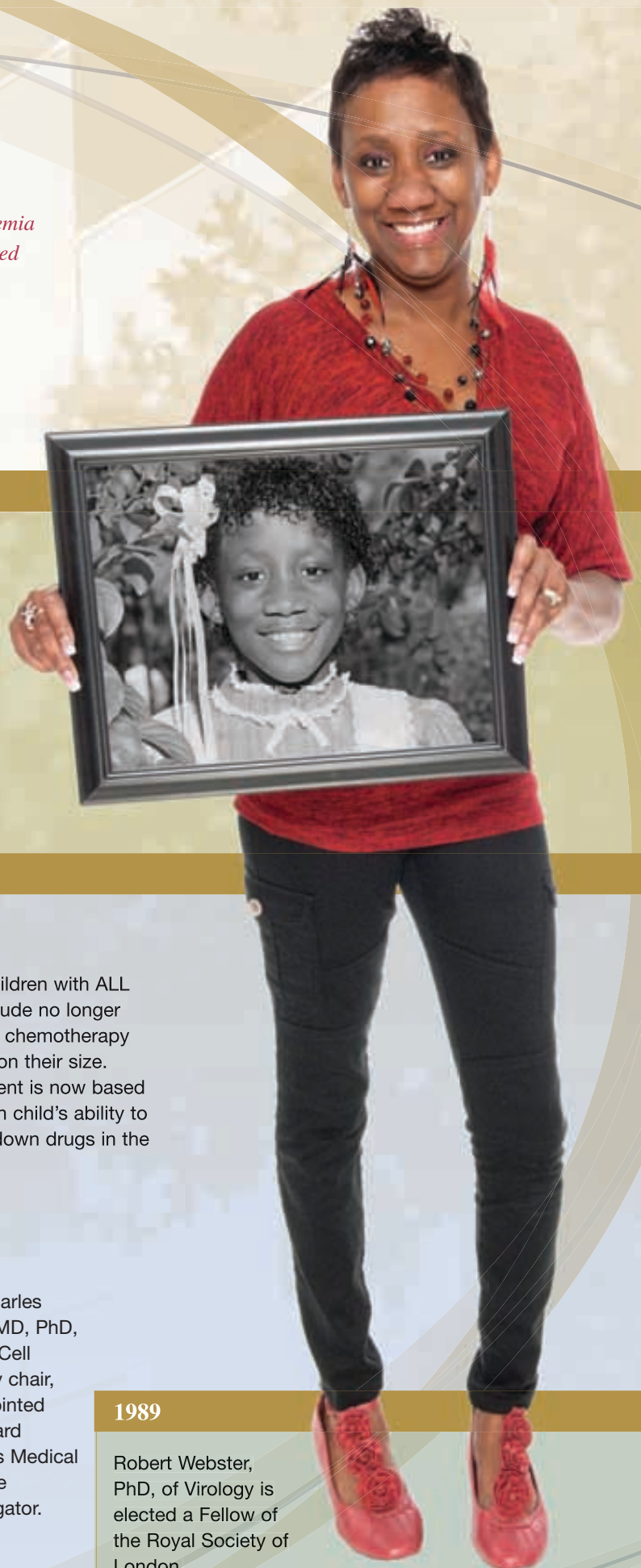
1986

The St. Jude Board of Governors declines an invitation to move St. Jude to St. Louis, Missouri. Afterward, the hospital has a renewed sense of resolve to fulfill its potential.

Using risk-directed therapy, clinicians begin reducing the amount of cranial irradiation for children with ALL.



*Kimberlin Wilson-George made medical history when she underwent a bone marrow transplant for acute myeloid leukemia (AML) and had a fortunate outcome: The procedure also cured her sickle cell anemia. "I tell people all the time that I am a walking miracle," Kimberlin says.*



Scientists find alterations in a gene that codes for a growth factor receptor of certain types of white blood cells. This helps transform normal cells into leukemia cells and is responsible for their spread throughout the body.

St. Jude launches a pilot study showing that chemotherapy can be added after surgery to delay irradiation in infants and young children with brain tumors. This allows the children's brains more time to mature before undergoing irradiation, thus reducing the side effects of treatment.

## 1987

Founder Danny Thomas announces that HIV/AIDS falls within the parameters of the St. Jude mission. As a result, the hospital institutes a clinical program to seek a cure for pediatric AIDS.



◀ Children with ALL at St. Jude no longer receive chemotherapy based on their size. Treatment is now based on each child's ability to break down drugs in the body.



◀ Charles Sherr, MD, PhD, Tumor Cell Biology chair, is appointed a Howard Hughes Medical Institute Investigator.

## 1989

Robert Webster, PhD, of Virology is elected a Fellow of the Royal Society of London.

# 1990s

*"The greatest reward is the smiles on the faces of the children whose lives you've helped save."*

– Danny Thomas

1990



▲ Researchers determine that the risk of developing secondary acute myeloid leukemia (AML) is low or negligible in children treated for malignant solid tumors.

St. Jude clinicians successfully use radioactive implants to treat childhood brain tumors.

Blood cell growth factors are found to counteract life-threatening bone marrow depletion caused by the toxic effects of intensive chemotherapy.

St. Jude is the first to use gene marking to follow the course of bone marrow transplantation in children.

St. Jude scientists discover that an antimalarial drug can prevent or effectively treat a life-threatening form of pneumonia in patients with AIDS.

1991



▲ Danny Thomas dies February 6, two days after helping St. Jude celebrate its 29th anniversary.



◀ The survival rate for ALL reaches 73 percent.

The Danny Thomas Research Tower opens, with then-first lady Barbara Bush attending the dedication. ►





*Gabriela Salinas came to the United States from Bolivia in 1996, seeking treatment for a solid tumor known as Ewing sarcoma. At St. Jude, Gabby and her family found hope, healing and inspiration. Today, Gabby works in the hospital's Chemical Biology and Therapeutics department, giving back to the hospital that saved her life.*



By measuring the number of copies of the *N-myc* gene in neuroblastoma patients, St. Jude researchers individualize chemotherapy for those children.

The Ronald McDonald House of Memphis opens, providing housing for St. Jude patients and their families. ▼



Mammalian G1 cyclins (types of proteins involved in initiating cell cycles) are identified and associated with the development of certain cancers.

1992

Scientists identify key genes involved in two immunodeficiency diseases in male children, which creates a major contribution to the knowledge of hereditary disorders.



◀ St. Jude forms a Pediatric AIDS Clinical Trial Unit with two other Memphis area hospitals.

# 1990s

1993



▲ Arthur Nienhuis, MD, becomes the hospital's fourth director and CEO.

1994

A new and expanded Patient Care Center opens, with then-first lady Hillary Clinton as an honored guest. ▼



HIV infections are shown to be preventable by chemotherapy. The AIDS Clinical Trials Unit participates in a study showing that infants are at lower risk of acquiring HIV when ziduvodine, or AZT, is given to infected pregnant women and babies after birth.

Targeted T-cells are used as cell therapy against Epstein-Barr virus lymphoma.

1995

St. Jude is the first to adapt a computer-based, 3-D radiation therapy technique for pediatric brain tumor treatment.



▲ Cancer survival rates for African-American children are shown to have reached parity with Caucasian children when treated with protocol-based therapy.

Charles Sherr, MD, PhD, Tumor Cell Biology chair, is elected to the National Academy of Sciences.

1996

By opening vector production labs, St. Jude becomes one of the few centers in the world with a comprehensive cell and gene therapy program.

The world's first bone marrow transplant to treat osteogenesis imperfecta, a rare bone disease, is performed at St. Jude.



◀ Peter Doherty, PhD, St. Jude Immunology chair, is awarded the Nobel Prize for Physiology or Medicine. He and Rolf M. Zinkernagel, MD, PhD, of the University of Zurich share the prize for their pioneering research explaining how the immune system recognizes and kills virus-infected cells.



## 1997

Scientists demonstrate that bone marrow transplants from unrelated, genetically matched donors are as effective in treating childhood leukemia as those from patients' siblings who are genetically matched. ►

While identifying a new cancer-fighting tumor suppressor gene called *ARF*, scientists discover that a single genetic locus encodes protein products that regulate the most frequently targeted biochemical pathways in cancers.



▲ James Ihle, PhD, Biochemistry chair, is named a Howard Hughes Medical Institute Investigator.

## 1998

By individualizing the dosage of chemotherapy, scientists discover they can increase survival rates for children with ALL without causing excessive toxicity.

Investigators determine that the JAK-2 enzyme is essential to the production of red blood cells and platelets.

The survival rate for ALL reaches 80 percent.

Discovery of a new strain of drug-tolerant bacteria helps investigators take the first steps in developing a drug to eradicate antibiotic-tolerant bacteria and possibly antibiotic-resistant bacteria.

Peter Doherty, PhD, of Immunology and Robert Webster, PhD, of Virology are elected to the National Academy of Sciences.

## 1999



◀ Target House opens for St. Jude families requiring long-term housing.

A St. Jude study identifies a genetic defect that can predispose pediatric leukemia patients to develop secondary brain tumors. The investigators develop strategies to prevent this occurrence.

Scientists determine that the *Prox1* gene appears to play a primary role in lymphatic system development and might serve as a specific marker to analyze lymphatic system development.

Scientists discover a cellular reason why some cells are resistant to standard anti-HIV drugs.

Scientists find that a protein called BLNK is essential for normal development of the immune system.

St. Jude researchers discover why removal of two small enzymes called SOCS proteins can have deadly consequences. Advances in understanding the functions of the *SOCS1* gene, in particular, may have important clinical implications.

# 2000s

*"In a way, these findings represent coming full circle. St. Jude was the first to introduce cranial radiation as a treatment strategy that advanced the cure of childhood ALL to 50 percent. Now, St. Jude is the first to show that we can successfully eliminate irradiation by optimizing chemotherapy."*

*—Dr. William E. Evans, St. Jude director and CEO*

2000



▲ The Hartwell Center for Bioinformatics and Biotechnology opens. It integrates, in a single location, more state-of-the-art research technologies in computing and molecular science for the investigation of pediatric disease than any other such center in the world.

2002

The Institute of Medicine of the National Academy of Sciences elects its first members from St. Jude: then-hospital Director Arthur Nienhuis, MD, and William E. Evans, PharmD, who would become St. Jude director and CEO in 2004.

Scientists unveil a genetic screening technique using microarray chips that provides a new approach to diagnosing and treating acute lymphoblastic leukemia (ALL). The test is more than 95 percent accurate in diagnosing the known ALL subtypes and can identify new prognostic details.

The Integrated Research Center opens, housing 10 floors of research facilities.

St. Jude launches the *Cure4Kids* website. It provides clinicians worldwide with a free and open online meeting place for clinical discussions of childhood catastrophic diseases. ▼



2001



◀ The effect of radiation on a child's brain is measured for the first time in a pioneering study of pediatric brain tumor patients. The study enables doctors to plan radiation therapy so as to spare normal brain areas that could be negatively affected by radiation.

Researchers discover the world's first "universal" stem cell marker.

2003



▲ Using a reverse genetics system devised at St. Jude, scientists create a harmless version of avian influenza to be used as the master seed for vaccine manufacturing. The team produces a vaccine in only four weeks.



*Since arriving at St. Jude in 2009 with a severe form of acute myeloid leukemia (AML), 10-year-old Brennan Simkins has undergone four bone marrow transplants. "St. Jude has been a tremendous gift to our family," says his mom, Tara.*



2003



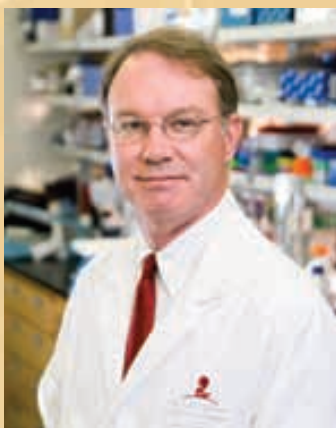
▲ St. Jude becomes the nation's first pediatric cancer research center to open a Good Manufacturing Practices (GMP) facility for producing vaccines, proteins, gene-based molecules and other biopharmaceuticals.

The hospital's sickle cell program is named one of 10 Comprehensive Sickle Cell Centers by the National Heart, Lung, and Blood Institute.

Investigators discover numerous genes that alter their level of activity in characteristic patterns in response to specific chemotherapy treatments. The genes are identified in the leukemia cells of children undergoing chemotherapy for ALL.

A St. Jude study comparing long-term outcomes of children treated for ALL shows that black children can do as well as white children if given equal access to the latest treatments.

2004



▲ Dr. William E. Evans becomes the hospital's fifth director and CEO.



▲ The Memphis Grizzlies House opens, providing short-term housing for St. Jude families.

A St. Jude study shows that conformal radiation kills tumors in children with the brain tumor ependymoma, while sparing normal tissues.

Investigators develop a laboratory model that closely mimics the human eye cancer retinoblastoma, giving scientists a way to test new therapies for this disease in the lab. ►

A study of children and adults finds that leukemic cells of each major known prognostic subtype of AML have a specific signature of gene expression.



Charles Sherr, MD, PhD, Tumor Cell Biology chair, is elected to the Institute of Medicine of the National Academy of Sciences.

Researchers find that by detecting the presence of the ERBB2 protein in tumor samples, doctors might be able to predict which children with the brain tumor medulloblastoma will require alternative therapies.

The ALL survival rate reaches 85 percent.

# 2000s

2005

Researchers show that certain traits inherited from parents can reduce the effectiveness of some chemotherapy drugs in children with ALL. This finding enables clinicians to identify patients at high or low risk of relapsing.



▲ Brenda Schulman, PhD, of Structural Biology and Genetics and Tumor Cell Biology, is named a Howard Hughes Medical Institute Investigator.

Thomas Curran, PhD, Developmental Neurobiology's founding chair, is elected a Fellow of the Royal Society of London.

Investigators discover that a specific pattern of gene expression in leukemic cells is linked to their resistance to anti-leukemic drugs. This finding helps to explain why standard therapies fail to cure about 20 percent of children with ALL.

2006



◀ St. Jude reports a 94 percent survival rate for patients with ALL, using therapy that does not include radiation.

St. Jude is named No. 1 on *The Scientist's* Best Places to Work in Academia list.

Improved treatment raises the survival rate of the brain tumor medulloblastoma to 85 percent for average-risk patients and 70 percent for high-risk patients. In average-risk patients, these rates are achieved while reducing the amount of radiation and length of chemotherapy following surgery. ▶



Researchers use gene expression profiling to discover five subtypes of medulloblastoma, which was previously thought to be one disease.

Scientists demonstrate that a new, locally applied treatment for the eye cancer retinoblastoma greatly reduces the size of the tumor without causing the side effects common with standard chemotherapy.

2007



◀ Researchers discover previously unsuspected mutations that contribute to the formation of ALL. The study generates worldwide excitement because it demonstrates a practical approach to screening large numbers of genes for mutations in order to identify unsuspected mutations in adult as well as pediatric cancers.

St. Jude is designated one of six Centers of Excellence for Influenza Research and Surveillance funded by the National Institute of Allergy and Infectious Diseases, a part of the National Institutes of Health.





▲ Investigators report the discovery of inherited variations in certain genes that make children with ALL susceptible to the toxic side effects caused by chemotherapy medications.

St. Jude researchers discover that a gene called *BRCA2* plays a dual role in the developing nervous system, eliminating errors in the DNA of newly made copies of chromosomes and suppressing the onset of medulloblastoma.

The St. Jude LIFE study begins. This initiative is one of the most ambitious follow-up projects ever conceived. It aims to bring thousands of St. Jude cancer survivors back to the place where they were treated as children to study the long-term effects of their disease and its treatment.



▲ Chili's Care Center, a state-of-the-art research and clinical care building, opens to house major research initiatives in diagnostic and therapeutic radiology.

Researchers show that a drug used for attention deficit disorder helps improve the attention, social skills and behavior of children treated for brain tumors and ALL.

Scientists discover that brain tumors arise from cancer stem cells that live within microscopic protective "niches" formed by blood vessels in the brain. Disrupting these niches is a promising strategy for eliminating the tumors and preventing them from re-growing.



▲ Investigators shed new light on why some children with an aggressive form of leukemia termed Philadelphia chromosome-positive ALL do not benefit from treatment.

Scientists identify the specific cell that causes eye cancer, disproving a long-held theory. Researchers find that certain mutations enable specific cells in the retina to multiply and cause retinoblastoma.

Investigators discover that a common housekeeping mechanism most cells use to keep their interiors healthy also helps immune system cells engulf and destroy germs.

# 2000s

## 2008

Kay Kafe, the hospital's renovated and expanded cafeteria, opens. St. Jude purposefully has just one cafeteria for all patients, families, physicians and staff.

Peter Doherty, PhD, of Immunology is elected to the Institute of Medicine of the National Academy of Sciences.

Investigators show how to predict if a child who is infected with respiratory syncytial virus (RSV) while being treated for cancer or another catastrophic disease is at high risk for developing severe infection.



▲ St. Jude physicians demonstrate that children with bilateral Wilms tumor, a cancer of the kidneys, can retain normal function in both kidneys by undergoing a procedure called bilateral nephron-sparing surgery, even when preoperative scans suggest that the tumors are inoperable.

## 2009



◀ Scientists identify distinctive genetic changes that cause relapse in children with ALL.

Investigators discover in children with ALL scores of inherited genetic variations that clinicians might be able to use as guideposts for designing more effective chemotherapy for this cancer.



◀ St. Jude receives a perfect score by the Joint Commission, a national organization that accredits health care organizations and programs in the United States.

Researchers identify mutations in the *IKAROS* gene that predict a high likelihood of relapse in children with ALL. By using a molecular test to identify this genetic marker, physicians should be better able to assign patients to appropriate therapies.





St. Jude is designated as a National Cancer Institute Comprehensive Cancer Center, making St. Jude the first and only cancer center solely focused on pediatric cancer to receive this distinction.

The first therapeutic monoclonal antibody produced by the Children's GMP, LLC, is approved by the Food and Drug Administration for use in clinical trials. The antibody is primarily produced to treat neuroblastoma.

Researchers find evidence that a series of genetic mutations work together to cause BCR-ABL1-positive ALL, an aggressive and often fatal form of leukemia. The researchers also find that loss of the *IKZF1* gene accompanies the transformation of chronic myeloid leukemias to a life-threatening, acute stage.



▲ Researchers discover that the drug amifostine is effective in preventing deafness in children with localized medulloblastoma.



◀ St. Jude announces that, with individualized chemotherapy, cranial irradiation can be totally eliminated from the treatment of children with leukemia.

The most comprehensive analysis yet of the genome of childhood acute myeloid leukemia finds only a few mistakes in the genetic blueprint, suggesting the cancer arises from just a handful of missteps.

◀ Nurses and staff in the Intensive Care Unit are recognized by the American Association of Critical-Care Nurses with the Beacon Award for Critical Care Excellence. They will win the award again in 2010.

Mary Relling, PharmD, Pharmaceutical Sciences chair, and Michael Kastan, MD, PhD, then-director of the St. Jude Comprehensive Cancer Center, are elected to the Institute of Medicine of the National Academy of Sciences.

*Parents* magazine names St. Jude as the nation's No. 1 pediatric cancer care hospital.

Scientists identify a subtype of acute T-lymphoblastic leukemia (T-ALL) that is resistant to standard chemotherapy. Clinicians plan to use this new insight to diagnose T-ALL and to use bone marrow transplantation to more effectively treat it.

St. Jude scientists identify inherited variations in two genes, *ARID5B* and *IKZF1*, that account for 37 percent of childhood ALL. Variations in *ARID5B* might also influence patient responses to chemotherapy.

Scientists who represent the hospital's interdisciplinary team studying ALL receive the American Association for Cancer Research's Team Science Award.

# 2010s

*"The St. Jude Children's Research Hospital – Washington University Pediatric Cancer Genome Project is the largest and most powerful single initiative in the history of St. Jude. This is an exciting time for St. Jude. But it's an even more exciting time for children worldwide."*

- Dr. William E. Evans, St. Jude director and CEO

## 2010



◀ St. Jude engages in the world's largest effort to identify the genetic changes that give rise to some of the world's deadliest cancers. The St. Jude Children's Research Hospital – Washington University Pediatric Cancer Genome Project (PCGP) holds promise for developing improved approaches to diagnosing, treating and perhaps even preventing certain cancers.

Survival rates for children with acute myeloid leukemia (AML) treated on a St. Jude protocol rise to 71 percent—20 percent better than previously reported U.S. rates.

U.S. News & World Report names St. Jude as the nation's top children's cancer hospital in its Best Children's Hospitals rankings.

The most comprehensive analysis yet of childhood high-grade glioma finds significant differences in the molecular features underlying the pediatric and adult forms of the cancer. Investigators identify a gene named *PDGFRA* as unusually active in some of the childhood tumors.



◀ Scientists discover that the subtypes of the brain tumors medulloblastoma and ependymoma arise from different types of brain cells.



◀ By screening a vast chemical library, an international team led by St. Jude identifies more than 1,100 compounds with confirmed activity against a deadly malaria parasite.



◀ Scientists find that patients in recent eras, who likely received treatments tailored to risk status, are not only surviving their cancer, but are also less likely to die later of treatment-related complications.

## 2012



PCGP scientists identify the mechanism that makes the childhood eye tumor retinoblastoma so aggressive and causes the tumor to develop so rapidly. The finding also leads investigators to a new treatment target and possible therapy.



*Just before Maggie Cupit was to embark on a college study program at St. Jude, doctors discovered that she had Ewing sarcoma. Instead of experiencing St. Jude as a student, she learned about it from a patient's perspective. "St. Jude is not a place of mourning," Maggie says. "It's a happy, positive place full of people all going through the same thing." She recently returned to the hospital—this time as a student.*



## 2011

St. Jude is named one of the country's "100 Best Companies to Work For" by *FORTUNE* magazine, a designation that is repeated in 2012.

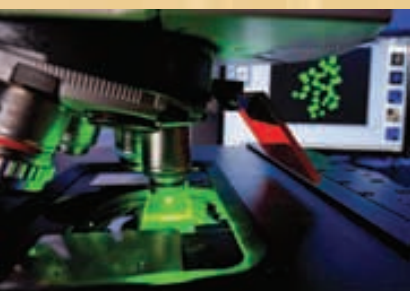
New research shows a drug commonly used to treat sickle cell anemia in adults reduces bouts of acute pain and a pneumonia-like illness, cuts hospitalization time and eases other symptoms of the disease in young patients.



◀ Bone marrow transplant survival more than doubles for young, high-risk leukemia patients treated at St. Jude, with patients who lack genetically matched donors recording the most significant gains. The results are believed to be the best ever reported for leukemia patients undergoing bone marrow transplantation.



◀ Investigators working on the PCGP develop a dramatically better computer tool for finding the genetic missteps that fuel cancer.



◀ Work led by St. Jude scientists pinpoints genetic factors that make Hispanic children more likely than those from other racial and ethnic backgrounds to receive acute lymphoblastic leukemia (ALL) diagnoses and to die of the disease.

Researchers tie a genetic variation characteristic of Native American ancestry to higher risk of relapse in young leukemia patients. Scientists find evidence that additional chemotherapy could eliminate the added risk.

For the sixth consecutive year, St. Jude is named as one of the top 10 institutions in the annual "Best Places to Work in Academia" list by *The Scientist* magazine.

Researchers identify promising new therapies for the brain tumor ependymoma. The drugs are found by screening 5,303 compounds for activity against the tumor.

A study of gene therapy developed at St. Jude and University College London offers first proof that the treatment benefits adults with hemophilia B, reducing the need for clotting factor to prevent bleeds.

PCGP investigators discover that early T-cell precursor ALL, a subtype of leukemia with a poor prognosis, is fueled by mutations in pathways distinctly different from a seemingly similar leukemia associated with a much better outcome. The findings highlight a possible new strategy for treatment.

PCGP researchers discover that nearly 80 percent of diffuse intrinsic pontine glioma tumors have mutations in genes not previously tied to cancer. The alterations may play a unique role in other aggressive pediatric brain tumors as well.

# Vision for the Future

*The true measure of our success will be our ability to give children the lives they deserve: that first step, first day of school, first date, first job. A lifetime of everyday moments.*



## A tradition of breakthroughs

In the 50 years since its opening, St. Jude Children's Research Hospital has led the way in transforming how the world treats pediatric cancer and other life-threatening diseases in children. Few organizations in the world are credited with as many paradigm-shifting discoveries as St. Jude. A group of St. Jude patients were the first children ever to be successfully taken off therapy for acute lymphoblastic leukemia. The hospital's researchers discovered that bone marrow transplants can cure sickle cell disease. St. Jude also pioneered personalized chemotherapy, eliminating the need for radiation therapy for the most common type of childhood cancer.

A logical question is, given all the progress that's been made, what could possibly be next?

The answer is simple. Danny Thomas once proclaimed that "no child should die in the dawn of life." Despite enormous progress in pushing overall U.S. survival rates from 20 to 80 percent, pediatric cancer remains the leading cause of death due to disease among U.S. children older than 1 year of age. Some types of pediatric cancer continue to have extremely poor survival rates, and the overall rate of improvement has slowed during the past decade. To further improve survival, big breakthroughs and innovative new treatments are needed; the kind St. Jude has proven it can deliver.





## From DNA to drug discovery

St. Jude is engaged in the world's largest project to sequence the complete genomes of pediatric cancer cells. This collaborative effort, called the St. Jude Children's Research Hospital – Washington University Pediatric Cancer Genome Project (PCGP), is generating a remarkable amount of new knowledge to drive improved diagnosis, treatment and perhaps even prevention of pediatric cancers.

Before this project, no one had sequenced a complete pediatric cancer genome, and yet scientists are on schedule to complete 600 sets within three years. Each set includes a complete genome sequence of tumor tissue, plus a complete sequence for normal tissue from the same child. By comparing the two, researchers hope to discover what causes a white blood cell to become a leukemia cell or a brain cell to become a brain tumor. St. Jude freely shares all data from this project as soon as the information has been validated and published.

The hospital continues to strengthen its capabilities for drug discovery, given the lack of financial incentives for pharmaceutical companies to discover new drugs for childhood cancers. Each year, 12,000 U.S. children—approximately 160,000 worldwide—are found to have cancer. St. Jude has stepped up to ensure that these children benefit from the remarkable advances in technology, high-throughput screening systems for new drugs, and next-generation genome sequencing. St. Jude recognizes that children need to benefit from these advances, even if no profit-based company will pursue it. Information coming out of the PCGP should create new targets for these drug-discovery efforts. The hospital is committed to paving the way for new drugs aimed specifically at childhood cancers.

## Creating long-term survivors

Treating childhood cancer today is not enough for St. Jude. Treatments developed and refined during the past five decades have created a new population that was almost nonexistent in

1962: long-term survivors of pediatric cancer. St. Jude is now studying long-term health outcomes of young adults who were cured at St. Jude of cancer 20, 30 or even 40 years ago, and is educating survivors about how to live healthy lives. These efforts are also helping us develop new treatments that are more effective and less toxic.

Just as the options for treating cancer today are much different than when we began 50 years ago, St. Jude is committed to changing them going forward, saving more lives and reducing treatment side effects.

The hospital is a national resource with a global mission. St. Jude discoveries help children around the world. Strategic relationships with organizations across the U.S. and abroad enable the hospital to even more quickly share knowledge and expertise to all corners of the world. For instance, the International Outreach Program helps more than a dozen developing countries increase and sustain access to modern treatments. The Cure4Kids Web-based educational program allows St. Jude to share critical knowledge and engage in real-time discussions with more than 30,000 health care professionals in more than 180 countries.

For researchers everywhere, the quest is survival for every child with cancer. St. Jude researchers will continue to chart new frontiers of discovery and innovation. The hospital's faculty and staff will continue to embody a culture of compassion, collaboration and innovation. St. Jude will continue to attract the best people and will provide an environment where they can do their best work.

The hospital's vision is unwavering: to make discoveries that save more lives; treatment advances that reduce side effects; and innovations that bring comfort, hope and support to families facing the toughest times imaginable.

The true measure of success will be the hospital's ability to give children the lives they deserve: that first step, first day of school, first date, first job. A lifetime of everyday moments. That is the legacy of St. Jude. ●

# A Legacy of Love and Hope

After more than a half century, millions of individuals continue to embrace one dream; one common goal.

BY LEIGH ANN ROMAN



*"NO CHILD SHOULD DIE IN THE DAWN OF LIFE."*

For more than 50 years, this deeply held belief of one devoted man has inspired and united people from all walks of life behind the mission of St. Jude Children's Research Hospital. Danny Thomas' vision and dream has become a rallying cry for people young and old.

**Business executives** such as Mike Tamer, a successful candy wholesaler from Indianapolis, responded to this plea from the heart. Tamer gave up his business to become the first national executive director of ALSAC, which united Americans of Arabic heritage to raise funds for the hospital's operating expenses.

**Children** such as Ann Hill answered Thomas' call. At 11, Hill raised more than \$1,100 for St. Jude with a lemonade stand. Although confined to a wheelchair because of a rare disease, she became the volunteer chairman for ALSAC in Mississippi, raising almost \$150,000 for St. Jude before her death at 25.

**Celebrities** such as Dinah Shore, Sammy Davis Jr. and Frank Sinatra embraced the dream, offering their talents to raise money at galas such as the St. Jude Shower of Stars. Today, Shaun White, Robin Williams, George Lopez, Jennifer Aniston and many other entertainers, actors and athletes carry on that legacy through the St. Jude *Thanks and*

*Giving*® campaign, which has raised \$312 million since it was launched in 2004 by Marlo, Terre and Tony Thomas, the children of Danny and Rose Marie Thomas.

**The everyday efforts of people nationwide** are integral to the hospital's success. Children who participate in St. Jude Math-A-Thon, college students who unite family and friends on behalf of the hospital through St. Jude Up 'til Dawn, and athletes who raise funds through the St. Jude Heroes program—all are making a difference.

St. Jude supporters respond to Danny's rallying cry in their own ways—whether they are retirees who give regularly as Partners In Hope, moms who are members of St. Jude partner Epsilon Sigma Alpha, or volunteers at the 34,000 fundraising events held each year. Each of these individuals keeps hope alive for children fighting life-threatening diseases.

"My father's legacy doesn't live in the bricks and mortar of St. Jude," says National Outreach Director Marlo Thomas. "What he built 50 years ago lives today in the precious boys and girls across this country and the world who are living happy, healthy lives because of our groundbreaking research that has led to pioneering treatment and care."

*Learn more about the St. Jude mission and how you can help at [www.stjude.org/waystohelp](http://www.stjude.org/waystohelp).* ●



# Celebrating 50 Extraordinary Years

Energetic entrepreneurs  
help hospital pursue Danny  
Thomas' dream.

BY JANICE HILL



**Danny Thomas' passion for children is reflected in a new generation of supporters, such as Bruce and Kathy Makowsky. After attending the hospital's Scientific Symposium, Kathy (pictured with St. Jude patient Anna Pike) commented, "I immediately felt that it was my destiny to help these children."**

Kathy and Bruce Makowsky, world-renowned handbag designers and importers, possess the dynamic, visionary energy of highly successful entrepreneur CEOs combined with heartfelt passion for the children of St. Jude Children's Research Hospital.

"I attended the Scientific Symposium a few years ago," Kathy says. "It was a time in our lives when we decided to wind down our careers as co-presidents of Van Zeeland Inc., and consider what we wanted to do next."

The symposium featured tours of the hospital and its long-term housing facility, Target House. Participants also heard from researchers and patient families.

"I fell in love with all of it," Kathy recalls. "I immediately felt that it was my destiny to help these children."

"I asked myself what could these families at Target House need that they don't already have? Since it was November, I asked if there was a wish list for Christmas gifts."

Kathy decided to fulfill the entire list. She contacted her sister businesses, and they all pitched in, providing all the gifts requested and much more. Kathy pledged to make it an annual tradition, and this year she expanded her donation to include the Ronald McDonald House of Memphis, one of the hospital's other residential facilities.

Kathy and Bruce's support extends far beyond the holidays, including generous outright gifts to the hospital, support for fundraising events and sharing the mission of St. Jude with others at every opportunity.

Kathy has returned to St. Jude several times. With each visit, her enthusiasm grows.

"My first visit was a life-changing day for me," she says. "It was a place of so much hope. What I learned about research was eye opening—for example, the fact that St. Jude customizes treatment for patients. Each visit teaches me more."

The Makowskys feel that St. Jude is a great match with their business philosophy.

"My husband and I are entrepreneurs in the true sense of the word," Kathy says. "We believe that if you have a good idea, anything is possible with passion and fortitude. I feel that same attitude at St. Jude, and it is the same passion that drove Danny Thomas to build the hospital 50 years ago. He had a dream that no child should die in the dawn of life—and I know that St. Jude will work until that dream comes true." ●

# Perspective

BY DR. WILLIAM E. EVANS

## From Dream to Reality

The hospital's director and CEO reflects on how the dream of one man transformed the world.

**H**ow did St. Jude Children's Research Hospital evolve from Danny Thomas' dream into a national resource that is now the world's top treatment and research center for children with cancer? During this milestone year for St. Jude, it is appropriate to pause and reflect on how vision, passion and partnership can change the world.

Like a great orchestra, St. Jude is the product of many inspired people working together, each bringing talents that are distinct and essential to the performance, yet doing so in a way that produces something magical and greater than the sum of the parts. I have been fortunate to witness this symphony for 40 of its 50 years, first in the balcony as a student, then in the orchestra section as a faculty member, and most recently on the conductor's stand. I have been lifted by each performance, regardless of my vantage point.

When the doors of St. Jude opened, most people thought its focus on childhood cancer was doomed to fail. Yet, a decade later St. Jude startled the medical establishment by introducing the word "cure" into the conversation about childhood cancer. At that moment, the medical world realized that something remarkable was happening in Memphis.

St. Jude represents one of the most unlikely yet powerful successes in academic medicine. If one were to pick a place to start a new biomedical research institute, Memphis in 1962 would not be near the top of the list. To launch a new "start-up" by giving away services for free would not be a business model found in any textbooks, then or today. And childhood cancers—largely considered incurable—would not have been the first choice for the focus of a new hospital. Yet Danny and his friends decided to do all of these things at once. The result was a new research hospital in Memphis, Tennessee, where no child would be turned away because of race, religion or the family's ability to pay.

Danny's dream was that "No child should die in the



**Dr. William E. Evans, St. Jude director and CEO**

dawn of life." This mantra, emblazoned on the name badge of every St. Jude employee, helps push the hospital forward 24 hours a day, seven days a week, 50 years later. Yet, despite the dramatic increase in survival rates, cancer remains the leading cause of death by disease in U.S. children over 1 year of age. Clearly, there is much more work to be done. Progress in our first 50 years has come from using drugs that were developed for adult cancers, not childhood cancers. Today, science and technology offer unprecedented opportunities to understand precisely what causes childhood cancers; from that will come new targets against which new medications can be developed. Our Pediatric Cancer Genome Project and our chemical biology efforts are our most recent new initiatives to build on our foundation of innovative clinical trials and cutting-edge laboratory research, and to find these new treatments for tomorrow.

St. Jude remains committed to leading this effort, so that 50 years from now we will be using medications that are less toxic and more effective in curing cancer—pushing us even closer to realizing Danny's dream. ●



# Saving children's lives

a legacy that stands the test of time

In 2002, when she was 8 years old, Caitlin started feeling sick. A CT scan revealed a tumor growing up her spinal canal. Caitlin was rushed to a St. Jude affiliate hospital, where she was diagnosed with Ewing sarcoma, a form of bone cancer. At St. Jude, Caitlin underwent chemotherapy, surgery and radiation. Early this year, she celebrated 10 years of being cancer free.

**Create a legacy gift that will help St. Jude continue its lifesaving mission so that one day children like Caitlin can realize their dreams.**



**Caitlin**  
age 17,  
Ewing sarcoma

Give a child hope  
for the future. Call today.  
**800.395.1087 | [www.stjudelegacy.org](http://www.stjudelegacy.org)**



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Tony Thomas discusses his father's legacy during the unveiling of a postage stamp honoring St. Jude founder Danny Thomas. Now on sale, the "forever" stamp commemorates Thomas' 100th birthday and the hospital's 50th anniversary.



*Finding cures. Saving children.*