

NATIONAL AND INTERNATIONAL RECOGNITION

St. Jude is leading the way the world understands, treats, and defeats childhood cancer and other life-threatening diseases. As we work diligently to accelerate progress and save lives, we are grateful for St. Jude to receive recognition from peers and partners.

Martine Roussel, PhD, a member of the Department of Tumor Cell Biology and co-leader of the Cancer Biology Program, was elected to the prestigious National Academy of Sciences. Dr. Roussel was recognized for her lasting contributions to the understanding of the molecular basis of pediatric brain tumors, and landmark discoveries in molecular oncology and translational treatment strategies for pediatric medulloblastoma, the most common malignant pediatric brain tumor. [Note: Medulloblastoma was the type of cancer Sam had.]

In February, St. Jude was named a recipient of Microsoft Corp.'s 2019 Health Innovation Awards for its work in creating the St. Jude Cloud, one of the world's largest repositories of pediatric genomics data. The awards recognize health organizations, like St. Jude, for using Microsoft's intelligent health technologies in innovative ways that help empower care teams, improve clinical and operational outcomes, and advance precision health care.

NEW PROGRAMS & INITIATIVES

St. Jude has acquired the most powerful superconducting magnet in the world – part of a new tool that will help researchers see farther into cells than ever before. The first Ascend 1.1 GHz Nuclear Magnet Resonance Spectrometer, the largest and most powerful device of its kind, is allowing St. Jude researchers to study proteins, DNA, RNA, and other biomolecules to better understand cancer and other catastrophic diseases that affect children. As part of our Structural Biology Department, this expansion has catapulted St. Jude as one of the world's premier centers for structural analyses and imaging of biological systems, with the goal of understanding health and disease at the molecular and atomic level.

In September, we marked the first anniversary of a vital online resource, *Together: Powered by St. Jude Children's Research Hospital*. To help meet the growing need for more resources for families facing childhood cancer, Dr. James Downing, St. Jude President and Chief Executive Officer, decided to build a website where families all over the world – not just those at St. Jude – could learn about pediatric cancer, its diagnosis and treatment, care and support, and life after cancer. People in 151 countries and all 50 states have accessed *Together*, which provides dependable information from a trusted source. Future plans include translating this information into more languages to reach even more people.

St. Jude was awarded a \$35 million grant for groundbreaking flu research. The National Institute of Allergy and Infectious Diseases awarded St. Jude the grant to direct the study, which involves investigators at 12 research and medical institutions in the U.S., New Zealand, Australia, and Nicaragua. Researchers hope the study will lay the groundwork for more effective flu prevention strategies, including possibly resulting in a universal vaccine to replace yearly flu vaccinations.

IMPORTANT SCIENTIFIC AND MEDICAL DISCOVERIES

Gene therapy developed at St. Jude has cured infants with X-linked severe combined immunodeficiency (X-SCID), also known as “bubble boy” disease. These patients are born with little or no immune system, and usually die early in life if not treated. The late Dr. Brian Sorrentino spent 25 years at St. Jude working on hematopoietic stem cell gene therapy for various diseases. He and his colleagues engineered modified cells for X-SCID patients at the Children’s GMP (Good Manufacturing Practices) facility on the St. Jude campus. Nine children received this therapy as infants and began producing fully functional immune cells for the first time. They appear to have been cured with no immediate adverse side effects, as detailed in the landmark St. Jude study published in the prestigious *New England Journal of Medicine*.

You may recall an important study called Genomes for Kids. I am pleased to share another significant finding related to the study, which revealed a gene commonly mutated in pediatric melanoma. Comprehensive clinical genomic testing of an adolescent patient helped researchers identify mutations in a single gene that drive the most common childhood melanoma. Further research found that 33 percent of young people with spitzoid melanoma in this study carried novel mutations, including rearrangements in the same gene, MAP3K8. Patients with this mutation may be candidates for precision medicine treatment. This finding helps further demonstrate the influence genomic research can have on understanding and treating diseases, which is why St. Jude performs comprehensive genomic tests on every new patient. More and more, we see this as a powerful tool to find cancer-causing mutations and spur development of precision medicines.

For the first time, scientists at St. Jude used nuclear magnetic resonance spectroscopy to map the structure of molecules called chaperones, which bind to proteins in cells to prevent them from malfunctioning. Reported in the journal *Science*, St. Jude researchers revealed how a chaperone attaches to a protein to protect it from going bad and causing disease. The findings help us understand how cells defend against mistakes in which proteins can unfold, misfold, or form toxic clumps. Before this work, scientists did not know the structural details of how chaperones linked with proteins.

UPDATES ON CAMPUS

We have recently welcomed two furry, four-legged colleagues to St. Jude. Puggle, a Golden Retriever and Huckleberry, a Golden Doodle, are part of a new program, St. Jude Paws at Play. The dogs are specially trained to help pediatric patients cope with the challenges of treatment and hospitalization. Among their daily duties are helping children achieve clinical goals, such as motivating them to get out of bed and walk after surgeries, or help prepare them for non-sedated scans. You can follow the daily work of Huckleberry and Puggle on Instagram: @stjudepaws.

In October, St. Jude hosted the 15th Annual Biomedical Research Symposium, bringing together leading experts in oncology, genetics, ethics, and computational biology. These experts discussed exciting new discoveries in the field of cancer predisposition and recently discovered cancer predisposing genes and associated genetic syndromes. Attendees also addressed questions and challenges that remain in the field.

When St. Jude's new Advanced Research Center opens next year, the center will speed discovery and transformative research, as well as attract more of the world's leading scientists to St. Jude. In addition, plans are proceeding for the Family Commons, the realization of a vision to expand our family-centered care for the wellbeing of our families – a place free from medicine and treatments, with fun and functional spaces to help children and parents focus on being a family.

YOU ARE PART OF THE CURE

As you can see, we have made great strides this year, but there is still so much work to be done. At St. Jude, we won't stop until no child dies of cancer. We are deeply grateful for your dedication, because it gives us the freedom to stay focused on our mission: Finding Cures. Saving Children.®

Together we can achieve extraordinary things for children battling cancer and other life-threatening diseases. From the bottom of my heart, thank you for all that you do for the kids of St. Jude.

With deep appreciation,
Richard C. Shadyac Jr.
President and Chief Executive Officer
ALSAC/St. Jude Children's Research Hospital