



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