



# TOGETHER, WE ARE CLOSER TO A CURE

## NEUROBLASTOMA

2019 IMPACT REPORT

Your support allows our world-renowned neuroblastoma experts at SickKids to actualize their most innovative ideas that will lead to better treatments for our patients.



# PIONEERING THERAPIES

Genetic sequencing and clinical trials are paving the way for better care.

**SINCE SEVENTY PER CENT** of paediatric illnesses have a genetic component to them, many patients could greatly benefit from treatments informed by precision medicine—a potentially less toxic approach for disease treatment and prevention that considers individual variability in genes, environment, and lifestyle. By sequencing a patient's DNA, clinicians and researchers gain critical insight into the genetic patterns of a patient's disease. A better understanding of neuroblastoma and the developments of new treatments hinge on advancements made in precision medicine and genetic sequencing, with multiple visionary research projects that are laying the groundwork for promising clinical trials.

## GENETIC BREAKTHROUGHS

In 2016, SickKids established the world-leading SickKids Kids Cancer Sequencing (KiCS) Program to improve outcomes for patients with relapsed or hard-to-treat cancers. The program is designed to analyze the genetic make-up of a child's tumour for a more accurate diagnosis and prognosis and better treatment with targeted therapies. Dr. Meredith Irwin, renowned oncologist and senior scientist in cell biology at SickKids, continues to spearhead the neuroblastoma initiative within KiCS, enabling 45 neuroblastoma patients' tumour and blood cells from SickKids and other Ontario sites to be genetically sequenced, informing new treatment options for patients. "A patient who failed to respond to conventional treatment for neuroblastoma is now responding to a targeted, precision therapy clinical trial after a mutation in their tumour was discovered through sequencing. We are hopeful for many more cases like this in the future," says Dr. Irwin.

Dr. Irwin and the KiCS team have already discovered new genes in neuroblastoma tumours that may be



Kathryn,  
neuroblastoma  
patient, SickKids

associated with the risk of developing an inherited form of the disease. Now, Dr. Irwin is determining how these genes operate and respond to therapies in the neuroblastoma cells to identify new targeted treatments for relapsed patients. The results of these studies have been presented at international meetings and may be the catalyst for promising clinical trials currently under development at SickKids.

## CLINICAL TRIALS TO TREATMENTS

SickKids runs some of the most promising paediatric cancer clinical trials in Canada. This year, Dr. Daniel Morgenstern, oncologist and Director of the New Agent and Innovative Therapy (NAIT) and Therapeutic MIBG programs at SickKids, continued to enroll patients in the New Approaches to Neuroblastoma Therapy (NANT) MIBG trial. SickKids is the only Canadian site to offer this innovative trial, which tests the efficacy of various combinations of drugs with MIBG therapy (targeted radiation therapy) against standard MIBG therapy in isolation. SickKids is also the only site for a cross-Canada NANT precision medicine trial using ALK inhibitors, a specific class of anti-cancer drugs, to target a gene mutation that occurs in 10 percent of neuroblastoma cases.

## NEUROBLASTOMA FACTS

WE CAN CURE ABOUT  
**45%**  
OF HIGH-RISK  
PATIENTS

AT DIAGNOSIS, OVER  
**1/2**  
THE CASES WILL  
HAVE METASTASIZED



## MORE MINDS. MORE OPPORTUNITY.

SickKids is home to leading researchers, clinicians and trainees from around the world who collaborate on visionary projects that lead to groundbreaking discoveries and cutting-edge therapies. This year, the Neuroblastoma Program welcomed Dr. Sarah Cohen-Gogo, the NAIT clinical research fellow working under the supervision of Dr. Daniel Morgenstern, and a graduate student and four summer students to assist in the study of neuroblastoma cells, determine the reason for their resistance to traditional treatments, and discover combinations of targeted therapies. Because of donors like you, we can help nurture the next generation of neuroblastoma experts who will continue the fight against neuroblastoma, and other cancers, at SickKids and around the world.

## STOPPING THE SPREAD

Studies aim to understanding why their cancer spreads, and how to halt it.

**METASTASIS IS VERY COMMON** in neuroblastoma cases. More than 50 per cent of patients are diagnosed once their cancer has already spread, and about 85 percent of patients relapse in the metastatic tumour site, and not the primary tumour site.

The prevalence of metastatic neuroblastoma, and the gap in research on the topic has led SickKids researchers Drs. Irwin and David Kaplan, senior scientist in Neurosciences & Mental Health at SickKids, to delve deeper into work on their first-of-its-kind neuroblastoma metastatic mouse model developed at SickKids.

While newly-diagnosed neuroblastoma is quite responsive to chemotherapy, the metastases that arise upon relapse are not. The key questions that they are asking are how and why the metastases arise from the original tumor, why those tumor cells thrive in the bone, bone marrow and brain, and why they are resistant to current treatment. Their goal is to identify therapies that will target metastases and eliminate them.

To achieve their goals, they are using a new technique called single cell sequencing that determines the makeup or molecular characteristics of each individual cell in the tumour and metastasis. They have found that some cells in the otherwise treatable tumour have the makeup of metastatic cells that will eventually travel to the bone and brain. Knowing the molecular "signature" of the metastatic cells has unveiled new ways to target and eliminate those often-fatal cells.

# THANK YOU.

Every day, SickKids is working towards improving the lives of children, and our success depends on the generosity of donors like you. Your commitment to the fight against neuroblastoma at SickKids ensures that we can continue to offer the best possible health outcomes for children around the world so they can live longer and healthier lives. Thank you for your remarkable generosity.



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