

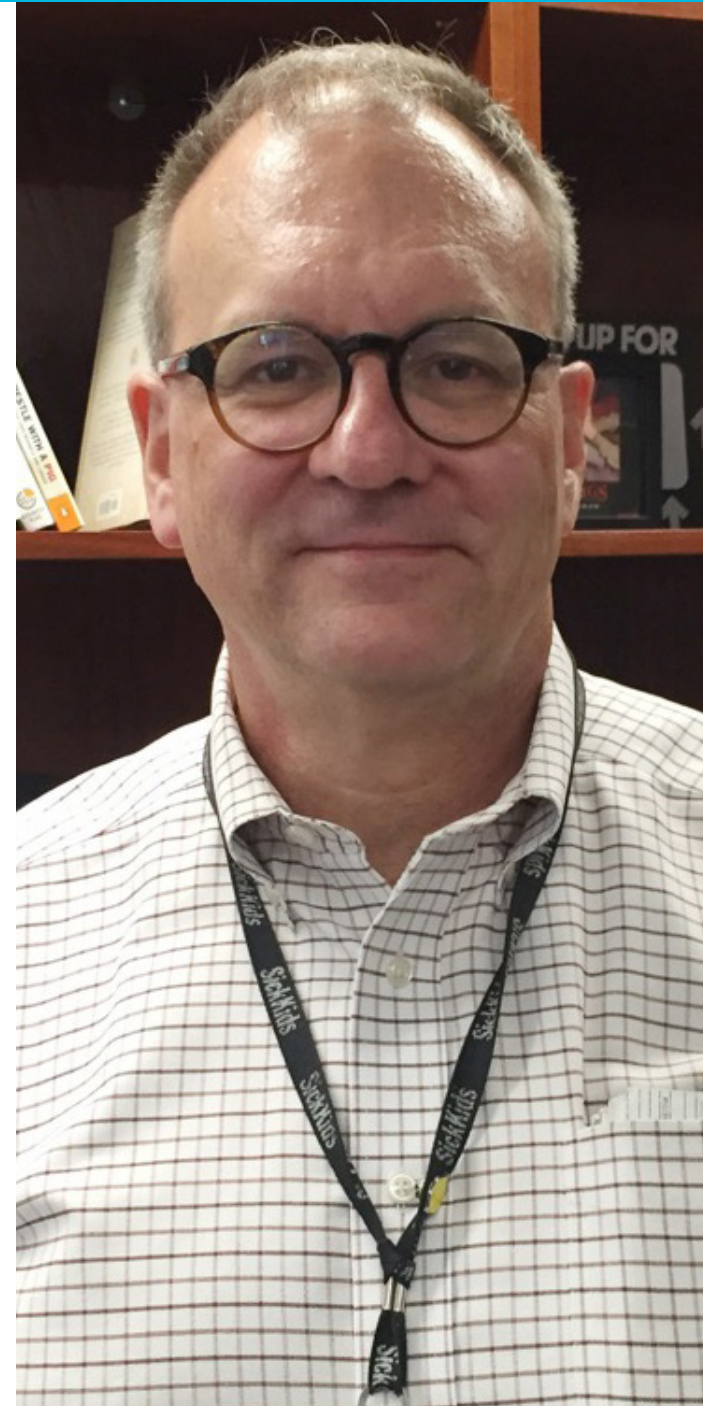


ANNUAL REPORT 2019-2020

SickKids® | Garron Family
Cancer Centre



MESSAGE FROM OUR CHAIR



Despite these challenging times, there still exists great innovation and opportunity in cancer research and care at SickKids, and the GFCC remains a strong and motivated leader in the field. As a virtual centre, GFCC continues to align multiple groups around shared priorities to provide life-saving and cutting-edge treatments. Our founding mission is as relevant as ever: to strengthen the integration of research, education and clinical care and foster innovation in cancer. In this 2019/2020 Annual Report, I am proud to present you with a review of what the clinicians, researchers and educators of the GFCC have accomplished this past year. In 2019 we released our [2020-2025 Strategic Plan](#). The priorities in this document highlight the direction of the GFCC, developed collaboratively through in-depth focus groups, online surveys and telephone interviews.

You will read about the psychosocial support infrastructure we are building for childhood cancer patients and their families; the research that is taking the most novel treatment paradigms—cancer precision child health and immunotherapy—to new heights; and the close collaborations we have developed with others across the hospital and research institute to enhance innovation. This important work reflects the Centre's larger collective focus: to help cancer patients not only live long but live well.

The GFCC is profoundly appreciative of the Garron family for their new gift of \$10 million over 10 years, beginning in 2021, which will support the Garron Family Chairs in Childhood Cancer Research and other high priority needs of the Centre. We are also grateful to the many patients and families from across Ontario and Canada whom our Centre is privileged to serve; to our devoted clinicians, educators and researchers who are dedicated to our vision, Better outcomes for children with cancer through multi-disciplinary collaboration, discovery and innovation. Thank you to our generous donors, and to our many members and supporters who are making the aspirations and achievements of the GFCC a reality.

Sincerely,

James A. Whitlock, M.D.
Director, Garron Family Cancer Centre
Division Head, Haematology/Oncology/BMTC
Women's Auxiliary Millennium Chair in Haematology/Oncology/BMT
Senior Associate Scientist, Translational Medicine Program

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MEET OUR HERO: Beckett Dougherty from Swift Current Saskatchewan. When Beckett was two and a half months old he developed what looked like a rash on his legs. Then the same rash appeared on his head. Beckett's mom took him to see his family doctor, expecting to be sent home with a prescription. Instead he was sent to a paediatrician and eventually to Saskatoon where a paediatric oncologist began treatment on his diagnosis: infant acute lymphoblastic leukemia. The oncologist in Saskatoon had done his Fellowship training in Toronto and recommended that he be transferred to SickKids as his case was extremely rare with only about ten cases a year being diagnosed across the country. After months of intensive chemotherapy in Toronto Beckett is now receiving ongoing treatment back home.

A YEAR LIKE NO OTHER

In the past year, the world as we know it has changed. But, the Garron Family Cancer Centre's (GFCC) commitment to support paediatric oncology patients in Ontario and across the country has not.

VISION, MISSION,
PRIORITIES AND GOALS

OUR MISSION

The mission of the GFCC is to facilitate and catalyze innovation in multi-disciplinary research, clinical care and education. We believe that discovery and translation of new knowledge will transform clinical practice and improve clinical outcomes and quality of life for children, adolescents and young adults with cancer and their families.

OUR VISION

Better outcomes for children with cancer through multi-disciplinary collaboration, discovery and innovation.

OUR PRIORITIES

The GFCC leadership has identified three strategic priorities that align with and leverage existing institutional and programmatic strengths. These priorities will guide the focus and investment of the Centre over the next 5 years.

- Improve the cancer journey through innovation
- Empower our people
- Optimize communication

OUR GOALS

The broad goals of the GFCC are:

- To elevate cancer care
- To deliver cutting edge innovative cancer therapies
- To create a more integrated cancer research community
- To support innovative cancer research
- To continue to develop excellence
- To support and retain excellence
- To strengthen communication with our community
- To strengthen partnerships for delivery of care

Read more about what these goals mean [here](#).



2019/20 BY THE NUMBERS (April 1, 2019 to March 31, 2020)

1/DAY
SickKids sees an average of
one new cancer patient every day

1400+
admissions per year

20,000+
clinic visits per year

>80% of children
diagnosed with cancer
at SickKids will survive

CANCER DIAGNOSIS

176
leukemia &
lymphoma patients

96
brain tumour
patients

151
solid tumour
patients

TRANSPLANTS AND CELLULAR THERAPIES

99
blood and marrow transplants
performed for patients with cancer

20
CAR-T
infusions

LEADERSHIP TEAM

EXECUTIVE COUNCIL

Brent Derry, PhD
Senior Scientist, Developmental & Stem Cell Biology

Meredith Irwin, MD
Chief of Paediatrics
Oncologist, Division of Haematology/Oncology/BMTC
Senior Scientist, Cell Biology

Jane McGlade, PhD
Senior Scientist, Cell Biology

Vijay Ramaswamy, MD
Oncologist, Division of Haematology/Oncology/BMTC
Scientist-Track Investigator,
Developmental & Stem Cell Biology

Judy Van Cleef, RN, BScN, MN
Vice President, Clinical

James Whitlock, MD
Director, Garron Family Cancer Centre
Division Head, Haematology/Oncology/BMTC
Senior Associate Scientist, Translational Medicine

Sue Zupanec, NP, MN
Nurse Practitioner, Division of Haematology/Oncology/BMTC

EX OFFICIO MEMBER(S)

Nancy Horvath
Director, Major Gifts, SickKids Foundation

Ulana Bochniewicz
Associate Director, Major Gifts, SickKids Foundation

PROGRAM MANAGERS

Donna Berry, PhD
Program Manager, Garron Family Cancer Centre
and Clinical Research Services

Kathryn Breckbill, PMP
Program Manager, Garron Family Cancer Centre
and Transplant & Regenerative Medicine Centre

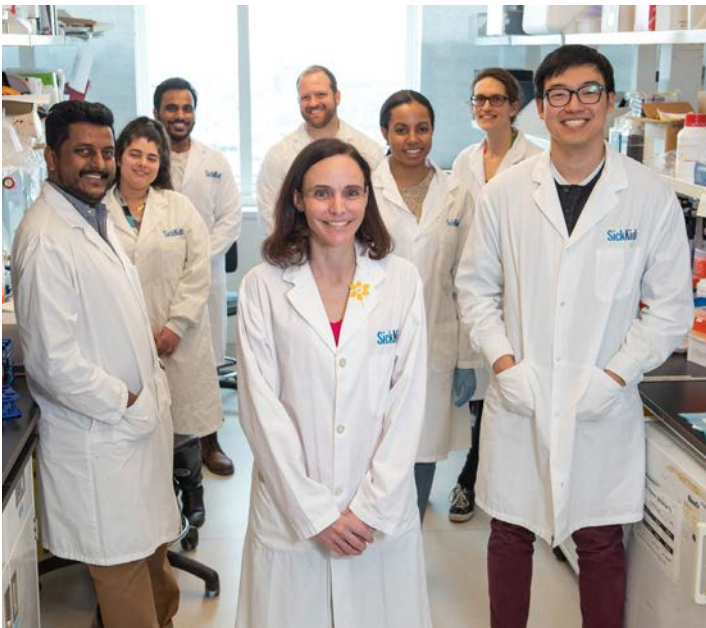
Devon Williams, PMP (Interim)
Program Manager, Garron Family Cancer Centre
and Transplant & Regenerative Medicine Centre

Thank you to the outgoing Executive Council Members
We extend our deepest thanks and sincere gratitude to **Dr. David Kaplan** and **Dr. David Malkin** for their wisdom and perspective that has been invaluable to the leadership team. The achievements of the GFCC are due in large part to their hard work and commitment. Their contributions have been significant and once again we thank them both for their service.

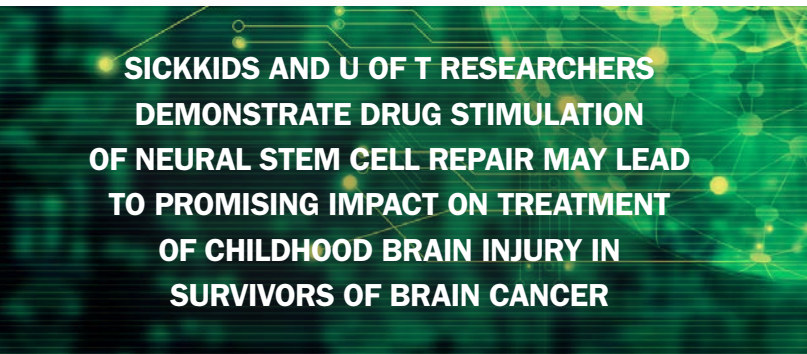
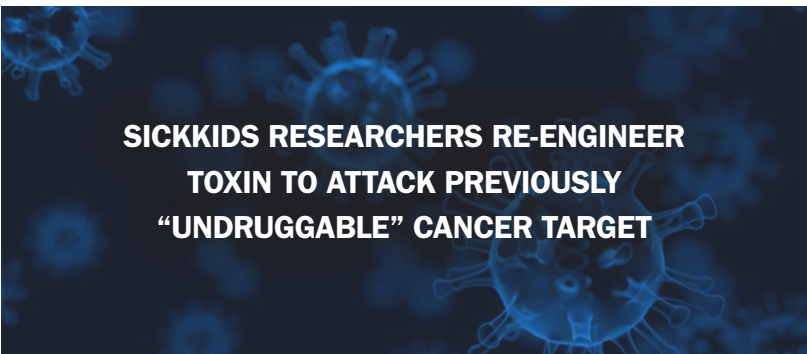
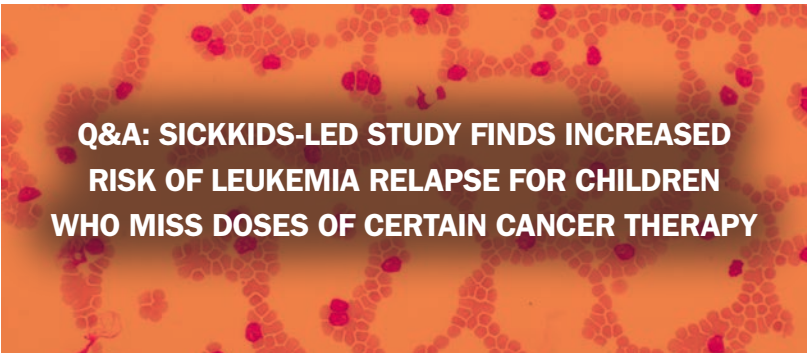
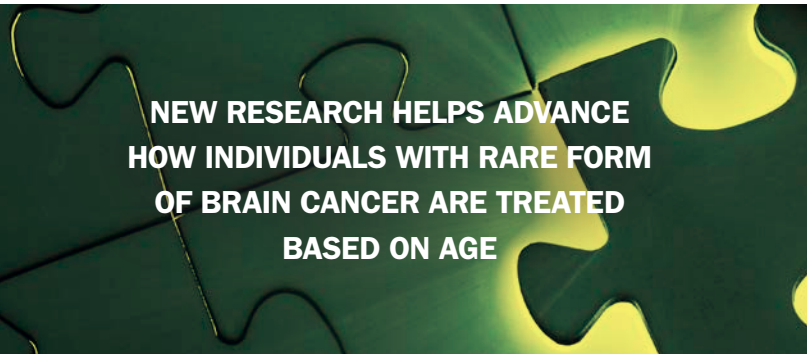
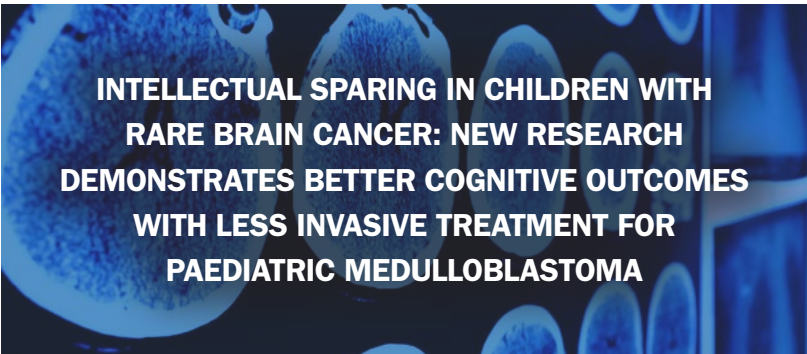
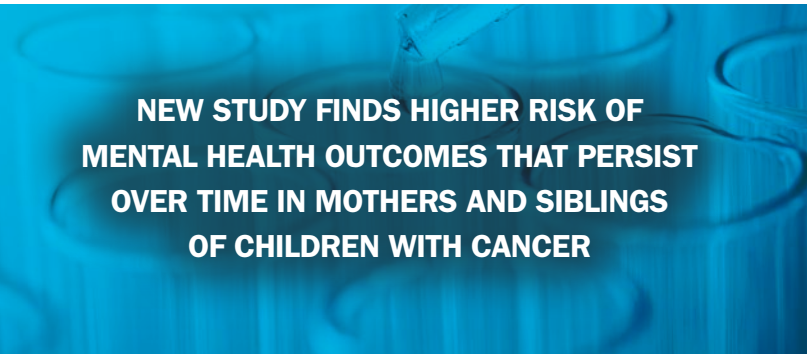
Welcome to the incoming Executive Council Members
The GFCC is pleased to welcome two new members to its Executive Council: **Dr. Jane McGlade** and **Dr. Vijay Ramaswamy**. These new members bring a breadth of expertise and experience that will ensure the GFCC is in a strong position to continue facilitating better outcomes for children with cancer. Welcome!

DR. CYNTHIA HAWKINS APPOINTED TO GARRON FAMILY CHAIR IN CHILDHOOD CANCER RESEARCH

Dr. Cynthia Hawkins is a Senior Scientist in the Cell Biology program and Neuropathologist in the Department of Paediatric Laboratory Medicine. Dr. Hawkins’ highly successful research program focuses on the molecular pathogenesis of paediatric brain tumours, as well as the development of novel prognostic and therapeutic markers for clinical implementation.



NEWS HIGHLIGHTS





IN LOVING MEMORY OF AMRITA NAIPAUL

It is with great sadness that the SickKids family said goodbye to Amrita Naipaul, Director, Haematology, Oncology, BMT and Palliative Care, unexpectedly on Aug. 13, 2019. Amrita joined SickKids in 2014 and has left an indelible mark on our organization, program, team and patients. She was a pioneer in the development of the first commercial CAR-T cell program in Canada. As a champion for patient safety, she was an early adopter and leader for the Caring Safely program, driving the division to have the largest number of active safety coaches. She was also a strong advocate for continuing education for nurses, supportive of opportunities for nurses to attend and present at conferences. Above all, she was passionate about creating and spreading joy, encouraging others to find their purpose in our work.

“

“Amrita was a vibrant member of our team. A strong, caring leader, and a passionate advocate for children with cancer and their families,” says Judy Van Clieaf, Vice-President, Clinical, and Chief Nursing Officer.

“We miss her drive and compassionate spirit, but most of all we miss her friendship.”



Ethan woke up on Christmas Eve too weak to get out of bed. He was rushed to SickKids where a diagnosis was confirmed: high-risk acute lymphoblastic leukemia. Ethan suffered several setbacks on his treatment journey until being put on an experimental immunotherapy drug to help fight the disease. The medication proved to be less toxic than conventional chemo and highly effective for Ethan. He is now on his way towards recovery.

**SUPPORTING
INNOVATIVE CARE**



COLLABORATIVE CANCER CARE - HAEMATOLOGY/ONCOLOGY PSYCHOSOCIAL PILOT PROGRAM

At the GFCC, we believe that every child and family affected by the devastation of a cancer diagnosis deserves timely access to the support they need to successfully navigate the journey ahead. Whether that be access to the best available medical care, information about resources needed to help with their journey, or help in dealing with the stress of a life-changing and life-threatening diagnosis. As a commitment to that vision, we are supporting the Haematology/Oncology Psychosocial program, led by Dr. Sarah Alexander, SickKids oncologist and clinical director of the Haematology/Oncology program, and Sonia Lucchetta, SickKids oncology social worker and Haematology/Oncology psychosocial program lead. This multi-faceted program strives to provide excellence in psychosocial care for all children and families affected by cancer and serious blood disorders. It's divided into four key priorities:

ONCOLOGY RESOURCE NAVIGATION

Families reeling from the shock of a cancer diagnosis often need help navigating their new reality. Wading through a maze of appointments, clinic visits and hospital stays can be exhausting and traumatic. Few have the time or energy to seek out resources to help lighten the financial, emotional and psychological burdens related to their medical care. The Oncology Resource Navigation Program is there to help.

Begun as a pilot program two years ago, it now sees more than 450 patient and family interactions per month and serves more than 300 new patients—ages 0 to 18—every year. The two-person team, made up of Lisa Berardo, social worker, and Denis Maplazi, child and youth worker, assists families with everything from looking for accommodations to filling out mountains of paperwork to finding financial support for new and routine expenses like wigs, travel to hospital, daycare, groceries, and so much more.

For particularly challenging cases, the team liaises with the larger Resource Navigation Service at the Hospital to access its vast support network and connects patients and families with specialized teams to address their mental health needs. This program has quickly become an indispensable part of the cancer care offered at the GFCC, helping to ease distress and smooth the treatment journey.

PSYCHOSOCIAL LEADERSHIP

Sonia Luchetta, SickKids social worker and program lead, has been integral in establishing well-attended interdisciplinary monthly team rounds, designing an invited speaker program, and being a source of team development, structure and guidance for the psychosocial program. Sonia's expertise, commitment to excellence, and overall unwavering leadership have been critical to ensuring the overall success of the program. "It is a privilege to be the first interim Haem/Onc psychosocial lead," Sonia explains. "Our group is comprised of experienced, compassionate and innovative practitioners. By coming together each month, we have been able to develop and implement creative programming for children and their families receiving care within our Division."

JOINING FORCES WITH PRINCESS MARGARET HOSPITAL

Cancer in children and adults is associated with enormous distress in both patients and their family members. SickKids and Princess Margaret Cancer Centre are both committed to family-based care, and are each recognized internationally for their excellence in the provision of paediatric and adult health care. Working together, the two institutions have piloted a new, integrated standard of psychosocial care for the patient and the entire family impacted by cancer diagnoses. Specifically, the joint program allows greater attention to the psychosocial needs of: adolescents and young adults with cancer aging-out of paediatric systems; caregivers of children and adolescents with cancer; and children of adults with cancer.

PSYCHOSOCIAL FELLOWSHIP

When a patient is diagnosed with cancer at SickKids, the whole family needs support. From a cancer diagnosis through treatment and all the way to recovery, Shawna Markowitz, social worker and fellow in oncology at the GFCC, is there to for the family – helping them face the psychosocial impact of their illness. "I work with leukemia, lymphoma and histiocytosis patients – helping them cope, process new information, access resources, or and anything else that comes up," Shawna explains. "You never know what the day will bring. I just adapt to their needs."

Shawna works as part of a wider team – collaborating with departments across the hospital – such as psychology, psychiatry, and Child Life services – to provide the best and most fulsome care possible to the whole family.

Shawna also takes an active role in developing, organizing and running educational sessions and workshops. With COVID-19 protocols now in place, she has made a quick pivot to adapt these sessions online. For example, a 90-minute online sibling session will be running this year, providing new diagnosis information and coping strategies to siblings of patients from 6 through 18 years of age.

Shawna's role has become integral to the success of our patients. "We help families shift from a place of immobilizing fear to hope. Being a social worker is about encouraging patients and families to find the strength to cope with cancer." This oncology fellowship role was so successful that Shawna has transitioned to a permanent staff position, supported by Carley's Angels, as a social worker for the department.

MANUFACTURING CAR-T CELLS

Immunotherapy harnesses the power of a patient’s immune system to fight disease. A specific type of immunotherapy, known as CAR T-cell therapy, is a treatment in which a patient’s T cells (a type of immune cell) are engineered in the laboratory to seek out cancer cells and destroy them. The GFCC is one of two paediatric cancer programs in Canada to offer approved CAR T-cell therapies to patients. To date, over 50 per cent of patients had a positive response to the treatment.

With the success of clinical trials at SickKids and other leading children’s cancer programs followed by the approval for CAR T-cell therapy by Health Canada in 2018, this treatment is now available for children, adolescents and young adults at SickKids with recurrent or refractory childhood acute lymphoblastic leukemia – kids like Marky. Ten years ago, Marky would have likely succumbed to his cancer after chemotherapy and a bone marrow transplant failed to cure his hard-to-treat leukemia. But this past year he underwent CAR T-cell therapy at SickKids. Today, he is finally back home with his family and enjoying being a kid again.



Marky, SickKids patient

CAC INNOVATIVE PROJECT FUNDING

The GFCC is committed to funding initiatives to support the Centre’s vision: Better outcomes for children with cancer through multi-disciplinary collaboration, discovery and innovation. The projects highlighted below describe the successful recipients of the 2019-2020 Clinical Advisory Committee (CAC) Innovation Funding competition.

NEEDLES DESIGNED TO MINIMIZE TISSUE DAMAGE FOR LUMBAR PUNCTURES AND SPINAL CORD CHEMOTHERAPY: ASSESSMENT FOR PRACTICE CHANGE

Chemotherapy administered to the spinal cord is a critical component of treatment for many childhood cancers. In paediatric acute lymphoblastic leukemia (ALL), for example, chemotherapy treatments administered by way of a lumbar puncture are given over the course of 2-3 years. In early and intensive phases of ALL therapy, lumbar punctures are frequent, often as frequent as weekly. A known complication and distressing side effect of lumbar punctures in the adult population is headaches; however the experience of headaches in children with ALL related to lumbar punctures is relatively unknown. Recently the use of needles designed to minimize tissue damage, or atraumatic needles, have been endorsed by Children’s Oncology Group as a way to reduce the occurrence of post-lumbar puncture headaches. Due to limited clinical information about post-lumbar puncture headaches in paediatric oncology patients, nurse practitioner Sue Zupanec and her team are working to generate new knowledge on the experience of post-lumbar puncture headaches in the paediatric population and assess the feasibility of practice change to minimize symptom distress.

HAEMATOLOGY/ONCOLOGY MENTORSHIP PILOT PROGRAM

In order to retain new nurses, especially in a challenging subspecialty such as paediatric oncology, strong support is critical to avoid burnout. Sheila Gandhi began piloting a mentorship program in September 2019 as a way to support new nursing staff. The aim of the program is to foster a collaborative learning culture, increase job satisfaction, boost engagement in the work environment, retain staff, improve performance and assist with succession planning. “A strong mentorship program, Gandhi says, offers benefits to the mentee, the mentor, the department and the organization as a whole.”

TEENS4TEENS: A NOVEL ONLINE PEER SUPPORT INTERVENTION FOR TEENS WITH CANCER DURING COVID-19 AND BEYOND

It’s hard enough being a healthy teen, let alone a teen with cancer. Imagine trying to accomplish the developmental milestones of adolescence – developing independence from parents, adopting peer codes and lifestyles, accepting one’s body, and establishing a personal and future career identity – if you have been diagnosed with lymphoma or sarcoma and need intensive treatment with chemotherapy, radiation and/ or surgery. This pilot project, led by Dr. Chana Korenblum, is targeted toward patients with cancer, aged 13-18 years, receiving oncology care at SickKids. The goals of this project are to reduce social isolation amplified by the COVID-19 pandemic and fulfill unmet informational needs related to disease management and treatment through a) a drop-in

Zoom Healthcare session led by a healthcare professional (30 minutes once a week for 6 months) and b) a private, confidential, monitored group chat following the session where participants can post questions or comments and engage with other teens.

UNDERSTANDING PREVALENCE AND CAUSES OF DISTRESS AMONGST HEALTHCARE WORKERS

Research shows that the majority of nurses and physicians caring for cancer patients face moral distress, commonly resulting from conflict with families and perception of futile interventions/unnecessary patient suffering. Moral distress has been associated with increased burnout and consideration of resignation. While tools have been created to measure moral distress, effective ways to reduce moral distress is lacking within the literature. This project, led by Dr. Natalie Mathews, aims to measure baseline moral distress levels and identify root causes on the oncology ward as a first step to creating innovation to decrease moral distress in healthcare providers.

U-LINK.CARE, AN ONLINE PLATFORM DEVELOPED SPECIFICALLY TO BETTER INFORM HEALTH CARE PROFESSIONALS, PATIENTS AND FAMILIES ABOUT AVAILABLE CLINICAL TRIALS FOR PAEDIATRIC ONCOLOGY PATIENTS IN CANADA

There is clear need for a user-friendly information source on early phase clinical trials available for both children, adolescent and young adult (CAYA) patients in Canada, both for clinicians and patients/families. This project, led by Dr. Sarah Cohen-Gogo, aims to improve information about and accessibility to early phase clinical trials for CAYA oncology patients in Canada through the forthcoming launch of the Canadian U-Link.care website, in partnership with the French initiative U-Link.eu.

“We’re building a multi-pronged approach so we can figure out how to stop cancer cells from dividing and how to keep them from spreading. That’s how to take advantage of novel treatments and move the bar on survival for these kids.”

– Dr. Cynthia Hawkins



TARGETED TREATMENT. PERSONALIZED CARE.

When patients are diagnosed with cancer, they often receive a one-size-fits-all treatment plan. Despite this, they can respond quite differently, and until recently, doctors didn’t know why. After decades of research, it’s now understood that the genetic profile of a patient’s tumour defines how their cancer grows and spreads. This information is used to identify and design new targeted therapies – the cornerstone of a new treatment paradigm at SickKids called precision child health. This means we use information about the patient’s genes, environment and lifestyle to prevent, diagnosis and treat disease.

In 2016, SickKids established the SickKids Cancer Sequencing (KiCS) program. This was an opportunity for SickKids to take advantage of our expertise in genetics and oncology and improve outcomes for kids with relapsed or hard-to-treat cancers.

The KiCS program was developed to analyze the genetic make-up of a child’s cancer to determine a patient’s unique genetic fingerprint, identify the cause of the tumour, gain information about the prognosis, and implement personalized treatments.

Current genetic testing typically involves examining one gene or a few genes at a time. KiCS uses a new kind of genetic test, next generation sequencing (NGS), to test the entire tumour genome at once. This test is more fulsome, precise and cost effective.

Together with [PROFYLE](#), led by Dr. David Malkin, Co-Director of the Cancer Genetics and KiCS programs at SickKids, KiCS matches nationally-collected sequencing data and potentially life-saving drugs.

Since KiCS began, over 350 cancer patients from SickKids, other centres in Ontario, across Canada and around the world have been enrolled in the program, with clinically relevant mutations found in almost 75 per cent of cases. Eleven clinical trials that use genomically-targeted drugs have opened at SickKids and enrolled 66 patients, including six from out of province.



20% SURVIVAL RATE FOR KIDS WHO HAVE RELAPSED FROM CANCER (THE ONES MOST LIKELY TO COME THROUGH KiCS)

10-25% OF PATIENTS HAVE A GENETIC PREDISPOSITION ASSOCIATED WITH THEIR CANCER

~\$10,000 IS THE COST TO SEQUENCE AND INTERPRET ONE CHILD’S DNA

50% OF PATIENTS WILL HAVE A CHANGE TO THEIR DIAGNOSIS OR TREATMENT BECAUSE OF KiCS

“Today, we can identify changes in the DNA of almost 75% of paediatric and young adult cancers, and treat about half of them with new, targeted drugs.”

WATCHING AND WAITING

For patients with Li-Fraumeni Syndrome, it's not a question of if they'll get cancer, it's a question of when.

At 29, Amy was diagnosed with thyroid cancer. At 36, she was diagnosed with incurable, stage four breast cancer. Her doctors weren't convinced this was just bad luck, and their suspicions were confirmed when genetic testing revealed that Amy had Li-Fraumeni Syndrome (LFS), a hereditary predisposition to cancer. At 38, Amy also underwent surgery for lung cancer, discovered using an early detection protocol developed at SickKids.

Patients with LFS carry a mutation in a key cancer-fighting gene called p53, which guarentees over a 90 per cent lifetime risk of developing cancer. In about 40 percent of cases, carriers are diagnosed with cancer before celebrating their 25th birthday. That's why Amy decided to have her two daughters tested, too. Unfortunately, one of her girls tested positive for the mutant gene. Genetic testing in high-risk patients highlights the importance of surveillance strategies for early tumour detection.

It also offers hope for the development of innovative gene-based treatments – not only for LFS patients, but for all patients with high-risk cancer susceptibilities.

Dr. David Malkin, Director of the Cancer Genetics program at SickKids and Principal Investigator of a multi-disciplinary team study LFS explains, “We’re picking up tumours early and intervening so that the likelihood of survival is far greater,” he says. “At the same time, using artificial intelligence to predict which LFS patients will get what type of cancer, and when.”

Amy’s daughter is now part of the surveillance program, undergoing blood work and ultrasound every three months, and an MRI once a year.



CANCER SLAM

In September 2019, the GFCC hosted an unprecedented networking event for the SickKids cancer community. The event, fondly known as Cancer Slam, was conceived by Drs. Jack Brzezinski and Michael Wilson after identifying a need for enhanced cross-collaboration across the cancer space. The event was pitched as an opportunity for scientists, physicians, nurses, allied health professionals, and trainees’ alike to share ideas, seek new partnerships, and uncover opportunities for collaboration. The inaugural event included attendees and speakers from a wide range of backgrounds and was perceived to be very valuable, informative and beneficial to both knowledge and practice. The SickKids cancer community is still buzzing with energy and is looking forward to the next Cancer Slam event!

THE LANCET ONCOLOGY’S COMMISSION ON SUSTAINABLE PEDIATRIC CANCER

In September 2017, experts from around the world convened at SickKids to undertake a comprehensive analysis of the global status of addressing childhood cancer care as part of The *Lancet Oncology’s* Commission on Sustainable Pediatric Cancer.

“It’s imperative that oncologists, institutions, and governments around the world work together to ensure children with cancer are afforded the same opportunity to survive no matter where they live,” says Dr. Sumit Gupta, Commission co-lead and co-lead of [Policy and Economics Research in Childhood Cancer \(PERCC\)](#) at SickKids.

In April 2020, the Lancet Oncology Commission [report](#) was released. The report estimates that there will be 13.7 million new cases of childhood cancer globally between 2020 and 2050. At current levels of health system performance (including access and referral), 6.1 million (44.9%) of these children will be undiagnosed and a further 11.1 million children will die from cancer if no additional investments are made to improve access to health care services or childhood treatment. Of this total, 9.3 million children (84.1%) will be in low- and middle-income countries.

The report’s authors call for the following six actions to implement effective childhood cancer services: (1) incorporate childhood cancers into essential benefits packages when expanding universal health coverage; (2) develop national cancer control plans and provide predictable financing to ensure the expansion of sustainable care for children with cancer; (3) eliminate out-of-pocket expenditures for childhood cancer services, to halt abandonment of treatment; (4) expand access to effective services for childhood cancers by establishing cancer networks; (5) invest in the development of cancer registries that incorporate childhood cancers; and (6) invest in research, development, and innovation.

The release of the Lancet Oncology Commission report is timed to support the ambitious target of the World Health Organization (WHO) Global Initiative for Childhood Cancer, which aims to increase the cure rate to 60% for all childhood cancer patients globally by 2030.



IMPROVING CHILDHOOD CANCER OUTCOMES WITH THE CLINICAL TRIALS SUPPORT UNIT

By Jessica Cabral, Intern, Communications and Public Affairs

The Clinical Trials Support Unit (CTSU) in the Division of Haematology, Oncology and Bone Marrow Transplant/Cellular Therapy, is one of the largest clinical research teams at SickKids. Comprised of 29 team members, the CTSU currently manages over 100 active clinical trials, in order to improve childhood outcomes and provide the best possible treatment options.

The CTSU team makes every effort to enrol as many patients as possible in a clinical research study or therapeutic trial. In the 2019-2020 fiscal year, there were 343 oncology patients newly diagnosed with cancer at SickKids. In 2019, 295 patients were enrolled in a study with 219 of these enrollments in a therapeutic clinical trial.

“Our goal is to have active clinical trials open at all times, so that when a patient walks through the door and receives a cancer diagnosis, they always have a clinical trial option available that is either standard of care or better than standard of care,” says Entela Zaffino, Manager, Clinical Trials Support Unit & Clinical Informatics, Haematology/Oncology. “Over the years, the standard of care and the treatment of cancer for paediatric patients have been improved through this model where frontline treatment is also part of a clinical trial.”

During a typical day, the roles and responsibilities of the CTSU team members widely vary. From putting clinical trials through regulatory and ethics approvals to enrolling patients in trials to planning the logistics and operations of a trial, the CTSU is a one-stop shop for running clinical trials at SickKids.

The CTSU is supported in part by the Garron Family Cancer Centre.



RESEARCH GRANTS

BIG IDEAS IN FUNCTIONAL GENOMICS GRANT COMPETITION

In 2017, the GFCC launched a funding opportunity to support multidisciplinary teams in bridging the gap in knowledge between genetics and functional genomics in paediatric cancer. The primary aim of this funding is to enable teams of researchers at SickKids to leverage the latest research technologies to explore ideas that are “outside the box”.

In 2017, the first recipients were a team comprised of Drs. Brent Derry, Meredith Irwin and Michael Wilson to support a project entitled “Role of alternative polyadenylation in Ras-driven cancers.”

DNA is the blueprint the cell uses to make proteins, which are synthesized from an intermediate molecule called messenger RNA (mRNA). Defects in mRNA regulation that change how long their ends are can cause cells to adopt more tumour-like properties. Dr. Brent Derry’s lab uses a tiny nematode worm called C. elegans and human cancer cells to study how mRNA processing at their ends affects oncogene function. They discovered that when the ends of mRNAs are shorter cells become more tumour-like. Because there are hundreds of mRNAs affected in cancer they took advantage of the powerful tools of C. elegans to rapidly assess which ones were responsible for making cells more tumour-like through an oncogene called Ras. With funding from the GFCC Big Ideas in Functional Genomics grant, Dr. Derry’s team (led by postdoctoral fellow Dr. Aishwarya Subramanian) were able to identify two important genes (PAK1 and ABCC1), that amplify the oncogenic output when the ends of their mRNAs are shortened. To get a deeper understanding of how mRNA lengths are regulated and how this feeds the cancer cell, Derry’s lab teamed up with Dr. Michael Wilson and Dr. James Ellis at SickKids, as well as Dr. Mads Dugaard at the University of British Columbia. They are also working closely with Dr. Meredith Irwin and Dr. Adam Shlien at SickKids to ask if paediatric tumours, such as neuroblastoma, have similar defects in mRNA regulation. Given that all patients have unique genetic and genomic signatures Dr. Derry is hopeful their work will help them develop more precise therapeutic approaches for treating individual cancer patients in the future.

The GFCC funded two more exciting team projects in the 2019-20 competition:

Drs. Xi Huang and Michael Taylor – “Treating the Untreatable: Targeting Paediatric Brain Tumors Using Multifunctional Genomic Models”.

Drs. Sean Egan, Hans Hitzler, Cindy Guidos – “Modeling Pediatric Tq-Myeloid Leukemia”.

PITBLADO DISCOVERY GRANT COMPETITION

With financial support from the David Pitblado Cancer Research Fund, the GFCC holds an annual grant competition to support cutting edge research at SickKids. These one-year funding awards provide researchers the opportunity to explore creative ideas to advance our fundamental understanding of cancer and improve treatment outcomes for our patients. The success of this long-standing competition has allowed the GFCC and SickKids Foundation to leverage funds from other generous donors and expand the program’s impact. This year, through support from the David Pitblado Cancer Research Fund, Friends Helping SickKids, the Mirvish Family Oncology Research Fund, BGIS Research Endowment for Childhood Cancer and Fashion Heals for SickKids the GFCC was able to fund 5 innovative research projects:

Dr. Brenda Gallie – Retinoblastoma Activity Index (RAI): A Digital Solution to Standardize Outcome Measures in Clinical Trials for Intraocular Retinoblastoma

Dr. Sumit Gupta – The Risk of Late Effects Among Childhood Cancer Survivors with Down Syndrome: A Population-Based Study Using Health Services Data

Dr. Jane McGlade – Evaluation of TNIK Protein Kinase Role in Ph+ Leukemia

Dr. Vijay Ramaswamy – Characterizing Non-RELA Fusions of Chromosome 11 in Childhood Supratentorial Ependymoma

Dr. Rosanna Weksberg – Finding New Therapeutic Targets in Wilms Tumor Through an Integrated Analysis of Chromatin Structure – A Pilot Project

THE GREAT CYCLE CHALLENGE

The Great Cycle Challenge began in 2016 and after just four years has become one of the biggest cycling events across Canada. Every year the Great Cycle Challenge, in partnership with the SickKids Foundation, fundraises for the GFCC to fight kids’ cancer. These funds not only help SickKids patients but enables children across Canada to have access to better care. In August 2020, our community of over 52,206 pedaled a total of over 5.1 million kilometers raising funds in support of research to develop treatment and find cures for more children with cancer.



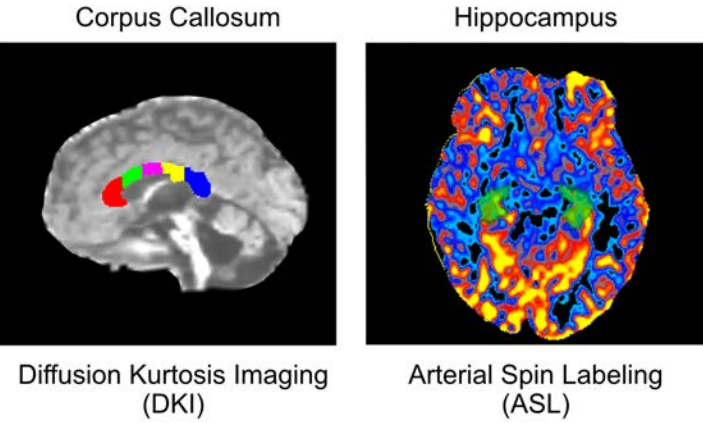
RESEARCH. TREAT. REPEAT.

Dr. Ramaswamy is getting better treatments to patients, faster. Dr. Vijay Ramaswamy’s research is focused on ependymoma and medulloblastoma – the first and third most common brain tumours in children. His goal is to move discoveries into the clinic quickly, then take those results back to the lab to improve treatments. Dr. Ramaswamy’s team is leading a clinical trial for recurrent ependymoma that’s been designed to reflect this back-and-forth continuum. With most trials, patients undergo surgery before receiving the trial drug to treat the recurrent cancer. In this case, patients receive the trial drug before a surgical procedure, a strategy termed iSpy in an innovative adult cancer clinical trial. It gives the team an opportunity to directly observe the effects of the medication on the tumour.

Dr. Ramaswamy has recently become an author on a clinical trial initiated when he was in high school. Here’s how: In 2004, as part of the Children’s Oncology Group, a clinical trial began for 325 children with ependymoma. But there were no stored tumour tissues from these patients to perform correlative studies which might explain these findings, since tissue banking as part of clinical trials wasn’t common practice then. Years later, Dr. Ramaswamy tracked down slides from the study that were used for alternative testing, and ran advanced genomic studies on the microscopic bits of tumour tissue preserved on them. “We took a study which was designed years ago with absolutely no biology in mind, and from discarded slides we were able to make a fairly big biological discovery. We now know which patients did really well on the drug, and which ones did poorly. And that saves us about ten years of work on how to advance treatment”, he explains.

RESEARCH PAPERS

ENHANCING BRAIN REPAIR FOR
BRAIN TUMOUR PATIENTS



Brain damage, secondary to cancer treatment, can have lasting effects on development and learning for children with brain tumours. Dr. Don Mabbott (Sickkids) and Dr. Freda Miller (University of British Columbia, formerly SickKids) developed a collaboration based on Dr. Miller’s discovery that metformin, an FDA-approved diabetes drug, could recruit stem cells to damaged white matter and stimulate repair. The team, which included Dr. Eric Bouffet (SickKids) and Dr. Cindi Morshead (University of Toronto), recently published a promising new study in Nature Medicine this past July, demonstrating positive effects of metformin on neurogenesis in an animal model. The results point to the exciting possibility that it might be possible to actively enhance recovery and brain repair using metformin. The study also evaluated the safety of metformin in human participants who had received radiation to the brain during treatment for brain tumours. Not only were no clinically relevant safety concerns identified, but a subset of the human participants were found to have improved performance on memory tests, when compared to placebo. This pilot trial, which was funded in part by the GFCC, is very encouraging and justifies a larger phase III study, currently in development. [Read more.](#)

NEW RESEARCH INFORMS HOW INDIVIDUALS WITH
RARE FORM OF BRAIN CANCER SHOULD BE TREATED



Dr. Annie Huang and Dr. Bryan Li

Brain tumours are the leading cause of paediatric cancer-related disability and death, and rare types of brain tumours make up approximately three to five per cent of those seen in children. To date, clinical studies of a highly cancerous brain tumour called pineoblastoma have been limited by the disease’s rarity and a lack of large, disease-specific cohorts to help inform new therapeutic approaches. Recent work led by Drs. Annie Huang and Eric Bouffet (SickKids) may have significant implications for the best treatment approach for pineoblastoma. The study, published in Acta Neuropathologica, represents a critical advancement in how individuals with this rare form of cancer should be treated based on their age group.

The study, co-authored by Dr. Bryan Li, a GFCC Fellow and clinician in the Haematology/Oncology Division at SickKids, presents the first molecular landscape picture of pineoblastoma, using integrated molecular and clinical analysis to describe five distinct sub-groups of the tumour. This exciting work provides a framework for treating each subgroup distinctly, to maximize effectiveness and limit unnecessary side-effects from aggressive therapy. Their findings have since been validated in a large collaborative meta-analysis with St. Jude Children’s Research Hospital and the German Cancer Research Centre and is being incorporated into the World Health Organization classification.

This work was made possible by the [Rare Brain Tumor Consortium \(RBTC\)](#), which Dr. Huang started at SickKids to advance research in rare childhood brain cancers for which there remains very little information to guide diagnosis and treatment. [Read more.](#)



SUPPORTING LEARNING
OPPORTUNITIES AND
CONTINUING EDUCATION

PROFILE OF GFCC RESEARCH FELLOWS



Dr. Sarah Cohen-Gogo,
Scotiabank Clinician
Scientist Fellowship

Supervisor: Dr Daniel Morgenstern, Staff Haematologist/Oncologist, Division of Haematology/Oncology; Director New Agent and Innovative Therapy program; Associate Scientist, Translational Medicine, SickKids Research Institute; Assistant Professor, Department of Paediatrics, University of Toronto

PARP inhibitors and chemotherapy for children, adolescent and young adults harbouring tumours with homologous recombination repair deficiency: a phase II pilot study

While the survival rate in Canada approaches 82% for all child, adolescent, and young adult cancer patients, the prognosis for patients with refractory, relapsed or metastatic disease remains unacceptably poor.

A pan-Canadian collaboration called PRecision Oncology For Young peopLE (PROFYLE) was created to improve survival for the patients with ‘hard-to-treat’ cancers by providing better access to next generation sequencing and targeted therapies. The PROFYLE program expands and integrates the Toronto regional paediatric cancer precision medicine program called Kids Cancer Sequencing (KiCS).

Among the identified genetic aberrations, those leading to deficiencies in a DNA repair pathway called homologous recombination are a promising group for targeted therapy. A family of drugs called PARP inhibitors is now part of the treatment for some adult cancers (ovarian, breast) that have such abnormalities.

As it remains unclear how to best identify paediatric, adolescent and young adult patients who may benefit from these drugs, we are developing a Canadian pilot clinical trial that will provide access to these new drugs for patients with hard-to-treat cancers, and take into account the information from the PROFYLE sequencing study to look for potential biomarkers.



Dr. Grace Egan,
GFCC Leukemia
Clinician Scientist
Fellowship

Supervisor: Dr. Aaron Schimmer, Senior Scientist, Princess Margaret Cancer Centre; Director, Research Institute, Princess Margaret Cancer Centre; Professor, Department of Medical Biophysics, University of Toronto; Staff Physician, Leukemia Site Group, Princess Margaret Cancer Centre

Nuclear hexokinase 2: mechanism of regulating stemness

Acute myeloid leukemia (AML) remains one of the most difficult to treat childhood cancers given its high rates of relapse and treatment-related toxicity. Leukemic stem cells (LSCs) are thought to be responsible for initiation, maintenance, and recurrence of AML. Understanding the biology of leukemia stem cells is crucial for developing effective, targeted, therapies for AML. Previous work has shown that enzymes involved in metabolism can influence gene expression when they move from the mitochondria to the nucleus. Little is known about this process in AML. Hexokinase 2 (HK2) is a metabolic enzyme involved in glucose metabolism. Recent work in our lab has demonstrated that leukemia stem cells have increased levels of HK2. When nuclear HK2 is over-expressed in AML cells, it is associated with a stem-like phenotype, making cells resistance to differentiation with chemotherapy. The mechanism of how nuclear HK2 contributes to stemness is unknown. My project will decipher these mechanisms, which appear to involve epigenetic processes and DNA damage repair signaling pathways. By exploring the interacting proteins and pathways of nuclear HK2, it may be possible to discover novel therapeutic ways of selectively reducing the leukemia stem cell population in AML.

PROFILE OF GFCC RESEARCH FELLOWS



Dr. Fyeza Hasan,
BMO Financial Group
Oncology Fellowship

Supervisor: Dr. Lillian Sung, Paediatric Oncologist, Division of Haematology/Oncology; Senior Scientist, Child Health Evaluative Sciences; Professor, Department of Paediatrics and Institute of Health Policy Management and Evaluation

Children with cancer as participants in end of life research

Survival rates for childhood cancers have increased dramatically, but 20% of patients still die of their cancer or its treatment. The end-of-life (EOL) care of these children is difficult and includes addressing complex symptoms, and challenging issues around decision making, such as decisions about resuscitation, ongoing therapy and clinical trials.

Palliative care research studies can help us to identify evidence informed strategies for managing the complex EOL issues faced by children with cancer and may improve quality of life and satisfaction with care for patients and their families.

Despite the need for this type of research, palliative care studies are extremely rare in paediatric oncology. There are a number of possible barriers including the reluctance of physicians to offer research to families, and reluctance of families to take part, or remain enrolled.

My research aims to explore stakeholder perceptions about the enrolment of children with cancer in paediatric oncology palliative care research. Studying the views of potential research participants will allow researchers to understand parents’ and clinicians’ decision making processes. This will assist them in designing studies that are meaningful and acceptable to stakeholders, improving recruitment and creating knowledge that ultimately improves EOL care.



Dr. Bryan Li,
BMO Financial Group
Oncology Fellowship

Supervisor: Dr. Annie Huang, Staff Oncologist, Paediatric Brain Tumour program, Division of Haematology/Oncology; Senior Scientist, Cell Biology; Principal Investigator, The Arthur and Sonia Labatt Brain Tumour Research Centre; Professor, Department of Paediatrics, University of Toronto

Genomic landscape of pineoblastoma

Pineoblastoma is a rare brain cancer affecting mainly young children. They are difficult to treat, with only half of patients surviving despite aggressive therapy. Little is known of pineoblastoma due its rarity and the surgical difficulty in acquiring enough of a sample for detailed analysis. Through the international Rare Brain Tumour Consortium, we have gathered and are analyzing the largest known collection of pineoblastoma samples. We are currently working to find what molecular factors drive these cancers to behave so aggressively. Our goal is to determine which of these can be targeted with current and experimental drugs, then validate them using cell culture and mice studies. Our findings will be used to design the next clinical trial for children with pineoblastoma to advance treatment and improve survival.

Dr. Li was awarded a 2019 Pediatric Basic Scientist Abstract Award for his research and was featured as a platform presentation at the 24th Annual Meeting of the Society for Neuro-Oncology in November 2019.

PROFILE OF GFCC RESEARCH FELLOWS



Dr. Reena Pabari,
Scotiabank Clinician Scientist Fellowship

Supervisor: Dr. Cynthia Guidos, Senior Scientist, Developmental & Stem Cell Biology; Scientific Director, Flow and Mass Cytometry Facility, SickKids Research Institute; Professor, Department of Immunology, University of Toronto

The use of high dimensional mass cytometry to detect and characterize relapse-originating cells in acute myeloid leukaemia

Patients with acute myeloid leukaemia (AML) have high rates of relapse, despite initial apparent responsiveness to chemotherapy in many cases. There is evidence that the relapsed disease derives from a population of leukemic stem cells that are present at the time of diagnosis. In patients with acute lymphoblastic leukaemia (ALL), flow cytometry is used to identify the persistence of leukemic cells (minimal residual disease) after induction

chemotherapy in order to guide and intensify treatment if needed. In contrast, AML is a heterogeneous disease that is difficult to characterize with routine flow cytometry. I will be using high dimensional mass cytometry to characterize and evaluate the relapse-originating cells in diagnosis-remission-relapse samples from patients with AML. Mass cytometry can profile up to 40 markers per cell and has a greater ability to characterize heterogeneous cell populations. Our goal is to be able to identify and monitor the relapse-seeding clones in patients with AML in order to better guide therapy and prevent further progression of disease.

“Our group is comprised of experienced, compassionate and innovative practitioners. By coming together we have been able to develop and implement creative programing for children and their families receiving care within our Division.”

– Sonia Luchetta

GFCC FAMILY AND EDUCATION EVENTS

CHILDHOOD CANCER AND SICKLE CELL DISEASE AWARENESS EVENT – SEPTEMBER 24, 2019

Patients and families were invited to take part in an event in collaboration with community partners to raise awareness of childhood cancer and sickle cell disease. This annual event provides an opportunity for community partners to network and connect with patients and their families while promoting awareness amongst the broader hospital community.

PHOTOVOICE – SEPTEMBER 23, 2019

Photovoice is a therapeutic photography program focused on creating a supportive peer-focused environment aimed at bringing voice to teens with varying cancer diagnosis.



12TH ANNUAL CHILDHOOD CANCER THERAPY UPDATE – FEBRUARY 5, 2020

This one day symposium brings together national and international experts to share leading practices and applied science. The aim of the event is to achieve a thought-provoking, relevant and scientific meeting to advance paediatric oncology care locally, nationally, and internationally. Over 140 healthcare practitioners attended this annual event.

SICKKIDS VS PODCAST

SickKids VS PODCAST takes you to the frontlines in the fight for child health. In Episode 2: Hypermutant Tumours, Drs. Eric Bouffet and Uri Tabori describe the research behind treating cancer patients with drugs that unleash the power of the immune system. [Click here](#) to listen.





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Cancer Centre