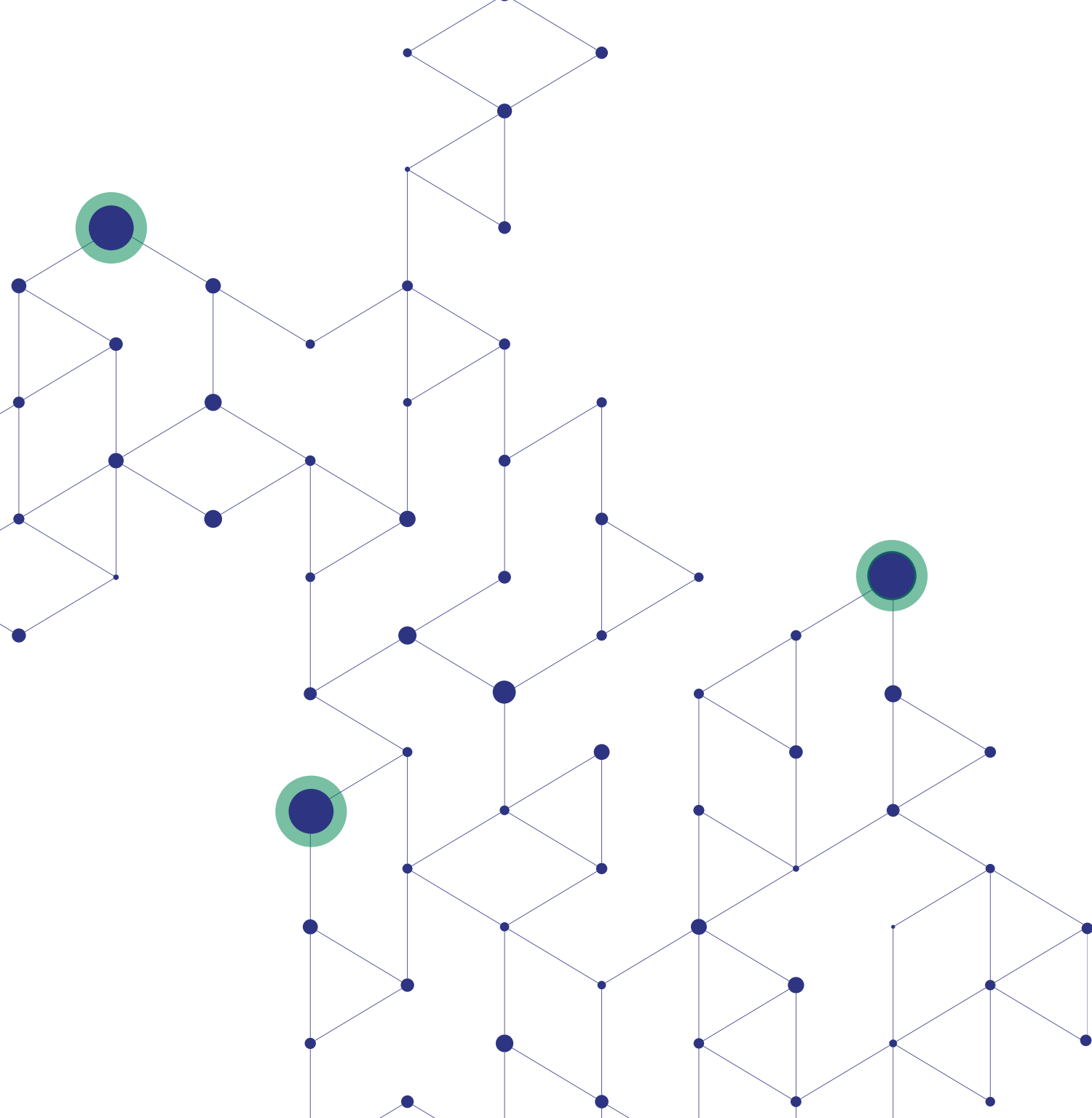


DEFINING MOMENTS

2019
ANNUAL REPORT



VISION

Discoveries today for healthier kids tomorrow.

VALUES

Excellence | Passion | Integrity | Creativity | Collaboration

MISSION

We conduct innovative research in a dynamic learning environment and translate that knowledge into practice and policies to improve the health of children, youth and their families.

CHEO Research Institute 401 Smyth Road Ottawa ON K1H 8L1 Tel: 613-737-7600
www.cheori.org



DR. JASON BERMAN
CEO AND SCIENTIFIC DIRECTOR
CHEO RESEARCH INSTITUTE

A SERIES MOMENTS

At its core, life is a series of moments. Some moments are deeply profound and alter one's direction, while others are more subtle and provide an opportunity to quietly reflect. There are also moments that shape us as professionals, and for me, this moment was joining the CHEO Research Institute.

As I anticipated a phone call about this new role on a cold January evening at the beginning of 2019, I contemplated the prospect of moving from Halifax to Ottawa. As many of you may have experienced, making decisions this significant that have both professional and personal implications are not easy decisions to make. I would need to take into account the feelings of my family, the impact on the members of my laboratory, and let's not forget about the many zebrafish! Upon this quiet moment of reflection, it became very clear to me that my decision was to proudly and delightedly accept the offer to join the CHEO Research Institute and the CHEO organization.

Thank you all for such a supportive and warm welcome. It has been extremely gratifying to witness and be a part of the positive energy throughout this organization. I especially appreciate your willingness to embrace new initiatives, as we begin to build our strategic plan to move this organization into the next decade. Our team at the CHEO Research Institute is one of the most dynamic of which I have ever been a part. I can proudly say that you are a group of passionate professionals on both the research and administrative sides, and you never fail to amaze me with your

commitment and creativity to fulfill and advance our research mission. Our vast scope of research areas, ranging from state-of-the-art immunology and genomic wet lab research to the most complex cutting edge clinical trials, demonstrates the lengths that CHEO investigators will go to make discoveries to improve the lives of children.

Reflecting on 2019 is difficult right now. The COVID-19 pandemic has halted us all in mid-moment. The way we live and how we operate here at the CHEO RI is changing day by day as we try to figure out our "new normal." Now, more than ever before, it is important to reflect and celebrate research at CHEO. In my role as your CEO and Scientific Director, it is my honour to support and provide direction to all of our scientists, investigators, research coordinators and staff.

I am extremely grateful for the engagement of the RI Board, the CHEO Executive, the RI administrative team, our university partners and the entire CHEO research community, including the CHEO Foundation who have helped us to achieve incredible progress over this past year. In this wonderful summary and look back at 2019, we have chosen to highlight a wide variety of defining moments that include grant successes, fellowships, and even an Order of Canada! These accomplishments are the accumulation of years of hard work, determination, and collaboration. Together, we continue to make discoveries today for healthier kids tomorrow, one defining moment at a time.

DIDN'T MISS **A** BEAT

Looking back at our defining moments of 2019, there were many opportunities for advancement, and occasions of great pride. In addition to the stories you are about to read, there were many new research initiatives, and we continued to build on our solid financial base.

From a board's perspective, one of the most significant risks to an organization is with a change of leadership. We have sailed through that change without missing a beat. We have moved forward with our success with our research grants and many new initiatives like our joint strategic planning with CHEO. Dr. Jason Berman and his team have quickly come together to make this happen. Dr. Jason Berman has brought his curiosity, engagement, and passion, demonstrating he is an exploratory leader taking the Institute and his team to new heights.

Let's celebrate the hard work and continued achievements and discoveries of our exceptional team in 2019. The Board recognizes that 2020 has brought unprecedented challenges and that all of the researchers and research staff at the RI are letting their passion for research be their guiding value through this challenging time. We thank you.

We know that passion and drive will get us back on course as soon as possible in 2020 and appreciate all your efforts and patience in making that happen.

Caroline Somers
Chair, Board of Directors
CHEO RI

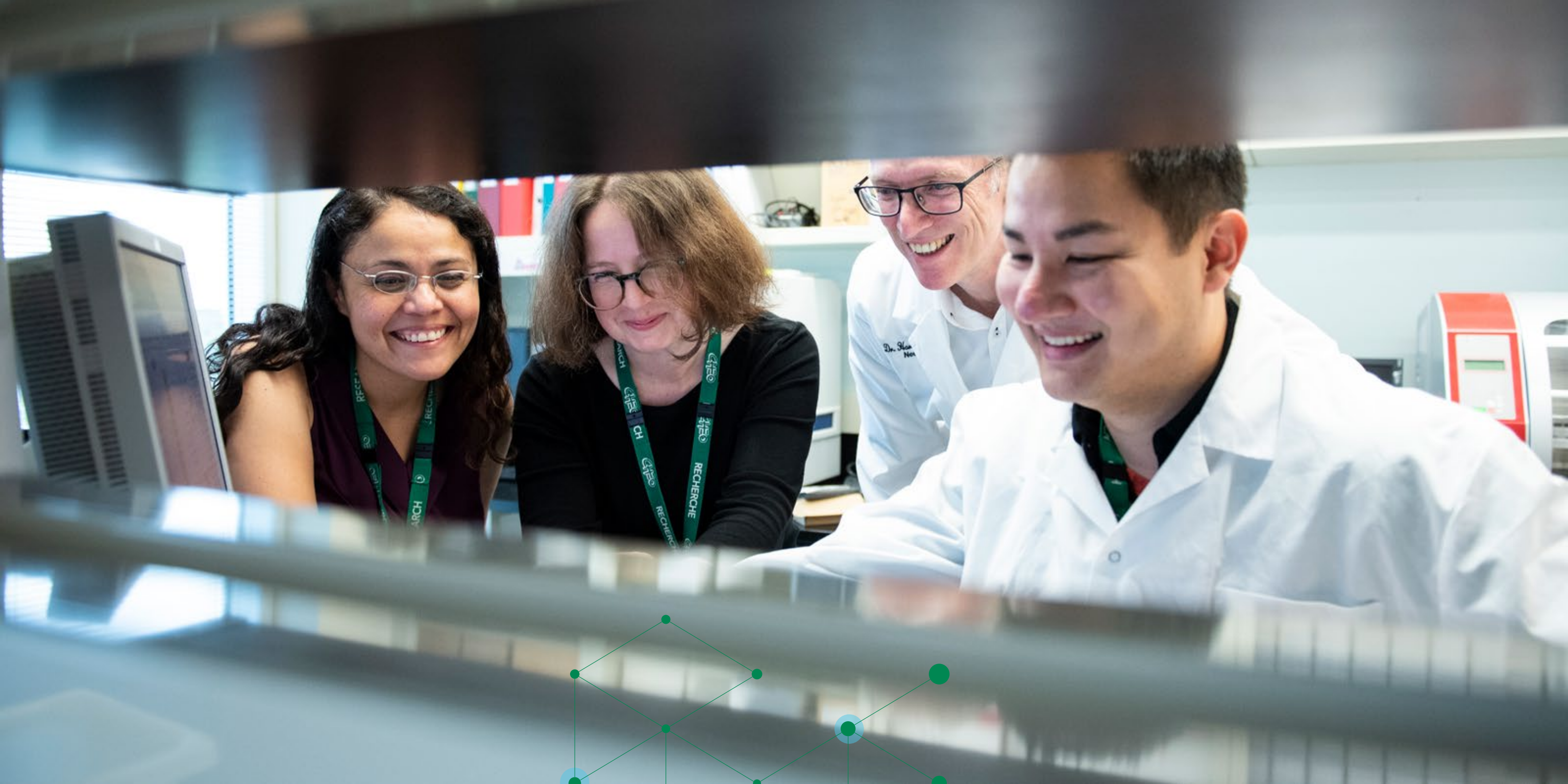


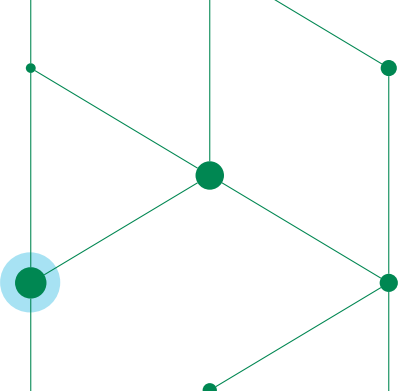
SUCCESS ON NEW SOIL

Grant success is often measured in dollar figures, yet the impact of a research grant goes far beyond the monetary award. When world-renowned Dr. Hanns Lochmüller was recruited to the CHEO Research Institute in 2018, he wasted no time recreating his successful research program on Canadian soil. Dr. Lochmüller, a neurologist whose research is focused on children with rare neuromuscular diseases (NMDs), received \$1.8M in grants this year. In addition, four of his lab members – Emily O'Connor, Rachel Thompson, Jarred Lau and Alex Manta – also received personal scholarships to support their work in his lab.

Dr. Lochmüller uses a signature 'bedside to bench and back again' approach to diagnose and treat NMDs. His approach is exemplified by his CIHR Foundation grant, Precision Health for Neuromuscular Disease, where he uses a variety of clinical and basic science methods to understand the molecular mechanisms of NMDs and work towards new therapies. Dr. Lochmüller says this grant was a defining moment for him and his







"I really believe working together helps us do better science. It's an honour to lead a fantastic group of neuromuscular clinicians and researchers from across the country to achieve better outcomes for patients by working together."

team. "As a new arrival in Canada, getting the CIHR Foundation grant was certainly a defining moment for me. It confirmed that moving my research across the world to join the CHEO Research Institute was the right choice. I've been made so welcome here by all my CHEO Research colleagues, and I feel tremendously privileged to have this stability of funding. It allows my lab to plan for a much longer-term than is typical, which helps us focus on the research questions we want to answer. I'm also incredibly lucky with the people around me – not only the excellent scientists I could recruit into the lab, but also support from key personnel such as Heather Howley the funding development officer at the CHEO RI who supported the application, and the many others in the institute who continue to support our work."

He also brings that collaborative leadership approach to CHEO Research Institute through his CIHR Network Catalyst grant, where he established and leads the Neuromuscular Disease Network for Canada (NMD4C), a new pan-Canadian network that brings together the country's leading stakeholders in NMD. "Receiving the NMD4C network grant was defining in a different way. It established my leadership role within collaborative science here in Canada. I've been passionate about sharing expertise and resources for many years – I really believe working together helps us do better science. It's an honour to lead a fantastic group of neuromuscular clinicians and researchers from across the country to achieve better outcomes for patients by working together."



A DEFINING **CANADIAN** MOMENT





NOVEMBER 21, 2019

Being acknowledged by your peers for your hard work is a source of pride for many in the scientific community. Having your work recognized by your country and given one of its highest honors, is an extraordinary moment, and one Dr. Robert Korneluk will not soon forget.

On November 21, Dr. Korneluk received the Order of Canada for his significant discoveries in understanding the factors that make human cells live or die. His innovative work using this knowledge to develop new therapies for cancer that harness the power of the immune system to target the cancer cell is world-class, with the potential to positively impact many patients affected with many different types of cancer. Being located at a leading research institute connected to a pediatric hospital enables Korneluk to collaborate with outstanding pediatric specialists. Meeting patients who have benefitted from his work reinforces his ongoing commitment to children's health.

Dr. Korneluk started his career at the CHEO Research Institute in 1985 and has been instrumental in some of the most important health discoveries made in the past three decades. He was a leader of the team that found the underlying cause of type 1 myotonic dystrophy (a disease that affects normal muscle function) in 1992. In 1995, Dr. Korneluk and his team discovered a family of genes that control apoptosis or cell death. This groundbreaking work has led to significant breakthroughs by Korneluk and other researchers around the world in areas such as cancer, diabetes, and neurodegeneration.

"Because of the discovery of the Inhibitor of Apoptosis (IAP) genes, we began to investigate their role in cancer immunotherapy with the goal to translate the findings into the clinic," said Dr. Korneluk. "Indeed, clinical trials based upon our immunotherapy approach are now ongoing in Ottawa and in other Canadian and global sites. I am especially proud of my group's efforts in translating these basic findings into promising, potential therapies for the benefit of cancer patients".

Governor General Julie Payette appointed Dr. Korneluk as a member of the Order of Canada. Dr. Korneluk is the very first CHEO researcher to receive this prestigious award. "I am extremely honoured to have been named as a Member of the Order of Canada," said Dr. Korneluk. "It's a moment I will never forget."

PAYING **IT** FORWARD



One of the areas where we have made the biggest strides over the last year at the CHEO Research Institute is partnering with patients in Research. Much of the work we are doing is trailblazing, and many other Research Institutes across the country have consulted with us when setting up their own research-patient partnership programs.

In 2019 we moved from engaging parents in Research to also partnering with our youth population that has seen research at CHEO change their lives. Currently, we have six youth members acting as partners in Research. Two members sit on our Patient and Family Advisory Committee and four younger members are part of a national committee that meets monthly. These energetic young people, advise from a youth perspective on issues like designing research data collection forms, providing input on the format of research ethics consent and assent documents, and establishing patient-centered research protocols and methodology.

When a child is diagnosed with an illness, it's a defining moment for them and their families. Through the Research Youth Advisory Committee, we hope to instill in these young people the difference research can make and how they share their treatment experiences can lead to new research and discoveries today for healthier youth tomorrow.

"I feel like I can learn new stuff and help other people that are in need," said Charlie Wingate, a 13-year old youth advisor. She has Cystic fibrosis and wants to make sure that her impact on research will improve the lives of other kids, "I feel that sharing my personal examples and stories will help make a difference and I am able to do that on this committee."

Young patients also act as ambassadors to raise awareness of the importance of partnering with youth on research projects. "Research means helping others with their medical conditions and making it easier to find treatments or a cure for their condition as well as making it easier to live with," said Jake Haydt, also 13 years old, and a committee member. "It is important to share the details of new research project ideas with the youth and give them the best understanding possible, not just their parents."



Jake has first-hand experience with many areas of research at the CHEO Research Institute. Having asthma, life-threatening allergies, and Attention Deficit Hyperactivity Disorder, he is empathic to other patients and families looking for answers in research. "I understand how kids and youth can sometimes have invisible challenges like ADHD, anxiety or learning disabilities, and I hope that my experiences and participation in this group can help them feel supported and feel like they're not alone. I like being a part of a research committee because I know that I am helping other children and their families get past hard and difficult times."

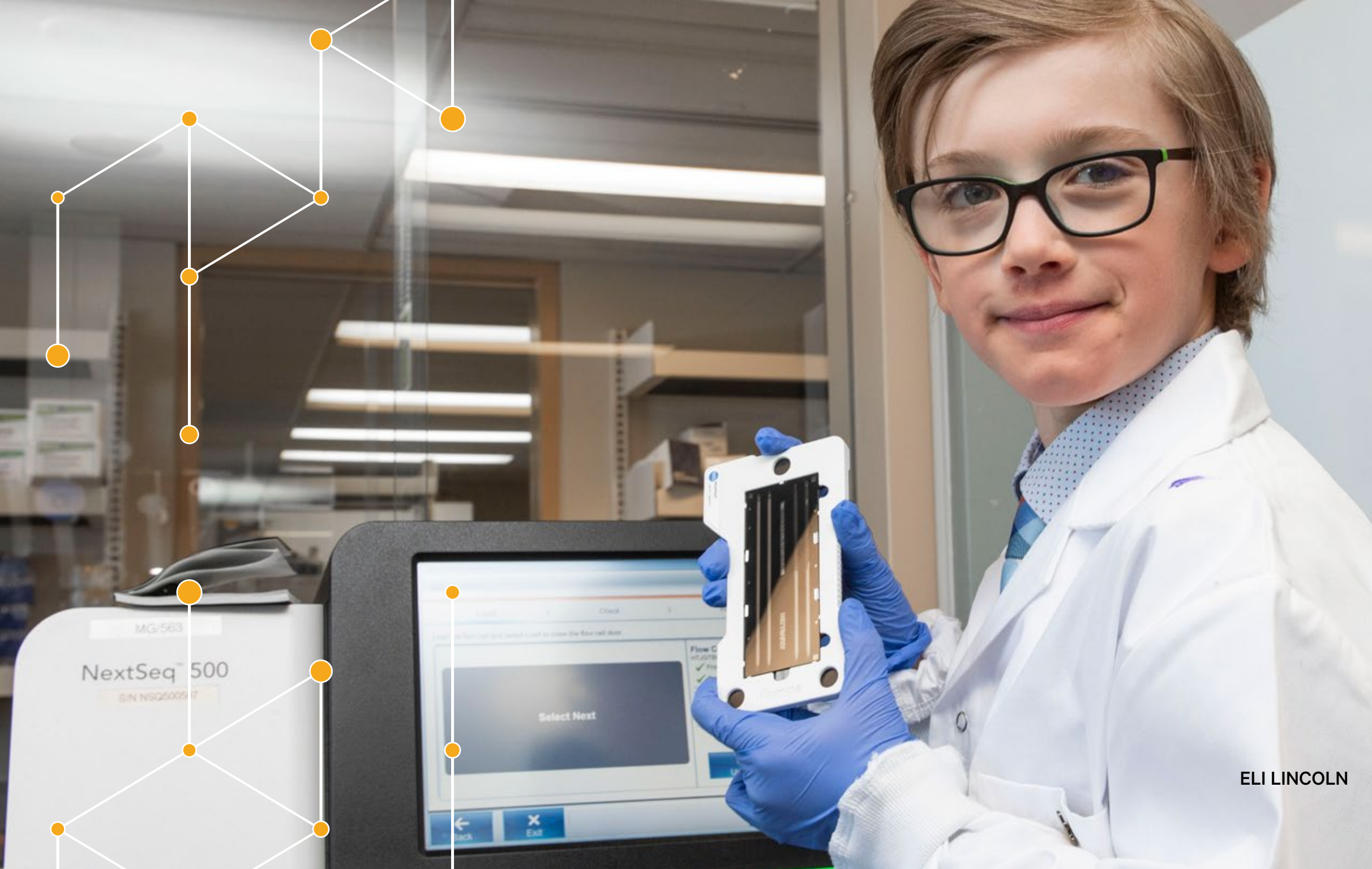


A RARE DEFINING MOMENT

On Rare Disease Day, February 28, 2019, CHEO and the CHEO Research Institute unveiled the Centre for Genomic Innovation. This new Centre, supported by the CHEO Foundation, delivers genomic discoveries to the front line of care for families with a rare disease. A first for Canada, the Centre catalyzes our international leadership in precision medicine for rare diseases to improve the lives of families locally, nationally and internationally. World-renowned Clinical Geneticist and Senior Scientist at the CHEO Research Institute, Dr. Kym Boycott, is the Executive Director of the Centre.

“Families with rare diseases often spend years seeking a diagnosis because their disease is not recognized as genetic, causing delays in testing and result interpretation,” says Dr. Boycott. “Through our pan-Canadian research partnerships, we’re using genomics to transform the diagnosis and treatment of rare diseases. However, it can take years for families living with a rare disease to receive the appropriate genomic test, and they wait far too long to get the answers they need. With this new investment in genomic innovation, we bring these genomic tests to the





ELI LINCOLN

front door of care – getting families the diagnoses they need when they need it. This is vital as an accurate diagnosis informs care, improves family well-being, and gives families access to resources and support.”

The Centre for Genomic Innovation helps health care providers to think differently, ask the right questions, expedite diagnosis and provide informed care. Dr. Boycott developed a first-in-Canada specialty clinic for families for whom genomic testing was appropriate. To expedite diagnostic testing, her research team identified appropriate families from the Genetics waitlist and redesigned how service was delivered. Families see Genetics earlier and get diagnostic results sooner. For families that receive a diagnosis, this clinic improves patient navigation by connecting them with appropriate specialists, clinical trials or approved therapies, and communities of support. To date, the Centre has made 135 new rare disease gene discoveries, and are currently working up an additional 116 potential discoveries. It is guaranteed that each discovery will be a defining moment in the lives of these children and their families.



CELEBRATING **IS** BETTER TOGETHER

For more than two decades, the research of Dr. Leanne Ward, a CHEO Research Institute Senior Scientist, has focused on children with rare bone diseases, and with bone morbidity due to serious illnesses. Last year Dr. Ward's defining moments were acknowledged on the international stage in two ways. First, she was elected Chair of the Scientific Planning Committee for the largest pediatric bone meeting in the world (The International Conference on Children's Bone Health). Second, she was one of the first and youngest recipients of a "Fellow of the American Society for Bone and Mineral Research (ASBMR) Award".

"My team at CHEO has worked diligently over the last two decades to develop new knowledge that optimizes the care of children with serious bone diseases," said Dr. Ward. "It means the world to us to know that our





peers recognize our work as having made important contributions to the lives of these children.”

Leanne and her team have made extraordinary contributions to the children’s skeletal health field through their research in pediatric osteoporosis and rickets (weak or soft bones). In their 2019 publication in *The Lancet*, Dr. Ward’s team worked with international partners to study a novel therapy for a rare disease called X-linked hypophosphatemic rickets. She and her colleagues discovered significantly greater improvements in growth and rickets severity for patients who were treated with a fully human monoclonal antibody targeting FGF23 (a hormone that is abnormally regulated in this disease), compared to conventional treatment.

Dr. Ward says a key component of her success as a physician-scientist is that 75% of her current research team has worked with her for over a decade, bringing to new projects all of the experience and skills of the prior work. “Sometimes the science limelight is focused on a person rather than teams. It’s important to me that we celebrate these moments as a team because we achieved them as a team.”

The CHEO Research Institute is proud of Dr. Ward’s accomplishments over the last year, and we look forward to her continued success.

DEFINING TEAMWORK

Informing the public and policymakers about the results of research is a vital element of the research process. When a team of researchers starts a new investigation, where that information will end up is often far from their mind. However, being published in journals like *The Lancet*, *The Journal of the American Medical Association (JAMA)*, and *The BMJ* can be an incredible defining moment for any investigator. This year the scientific team at Better Outcomes Registry & Network (BORN) Ontario not only had their findings published in each of these high impact journals, but they also made *JAMA*'s list of most talked-about articles of 2019.

The study, "Association Between Self-Reported Prenatal Cannabis Use and Maternal, Perinatal, and Neonatal Outcomes," was led by the team at BORN Ontario and published in *JAMA* on June 18th, 2019. The study found that among pregnant women in Ontario, reported cannabis use was significantly associated with an increased risk of preterm birth. "It's an honour to be published in The Journal of the American Medical Association in the first place," said lead author Dr. Daniel Corsi. "Making the most-read list was the icing on the cake and really demonstrates the value of BORN's data to researchers and policymakers."

Dr. Deshayne Fell, fellow author on the paper and Scientist at the CHEO Research Institute, has a theory as to why the paper was so widely read. "It was a defining moment for substance use research when cannabis became legal in Canada," said Dr. Fell. "People have made assumptions about the effects of cannabis use in pregnancy for years; now that it's legal, people want to replace those assumptions with evidence-based facts."

"There has always been a prevalent rumour that cannabis can curb morning sickness for expectant mothers," said Dr. Mark Walker, Scientific Director at BORN. "The uniqueness and the depth of the BORN dataset, combined with world-class methodology, is what made this study, and others we have published this year so well received."

This year the team also published "Health outcomes of young children born to mothers who received 2009 pandemic H1N1 influenza vaccination during pregnancy: a retrospective cohort study" in *The BMJ*; "Use of a maternal newborn audit and feedback system in Ontario: a collective case study" in *BMJ Quality and Safety*; and "A 2-year review of publicly funded cell-free DNA screening in Ontario: utilization and adherence to funding criteria" in *Prenatal Diagnosis*.

Having a child is one of the most defining moments in a parent's life. Through programs like BORN Ontario working in partnership with the CHEO Research Institute, we aim to provide parents and families with discoveries to make the best decisions they can during pregnancy.

DR. MARK WALKER



DR. DANIEL CORSI



DR. DESHAYNE FELL



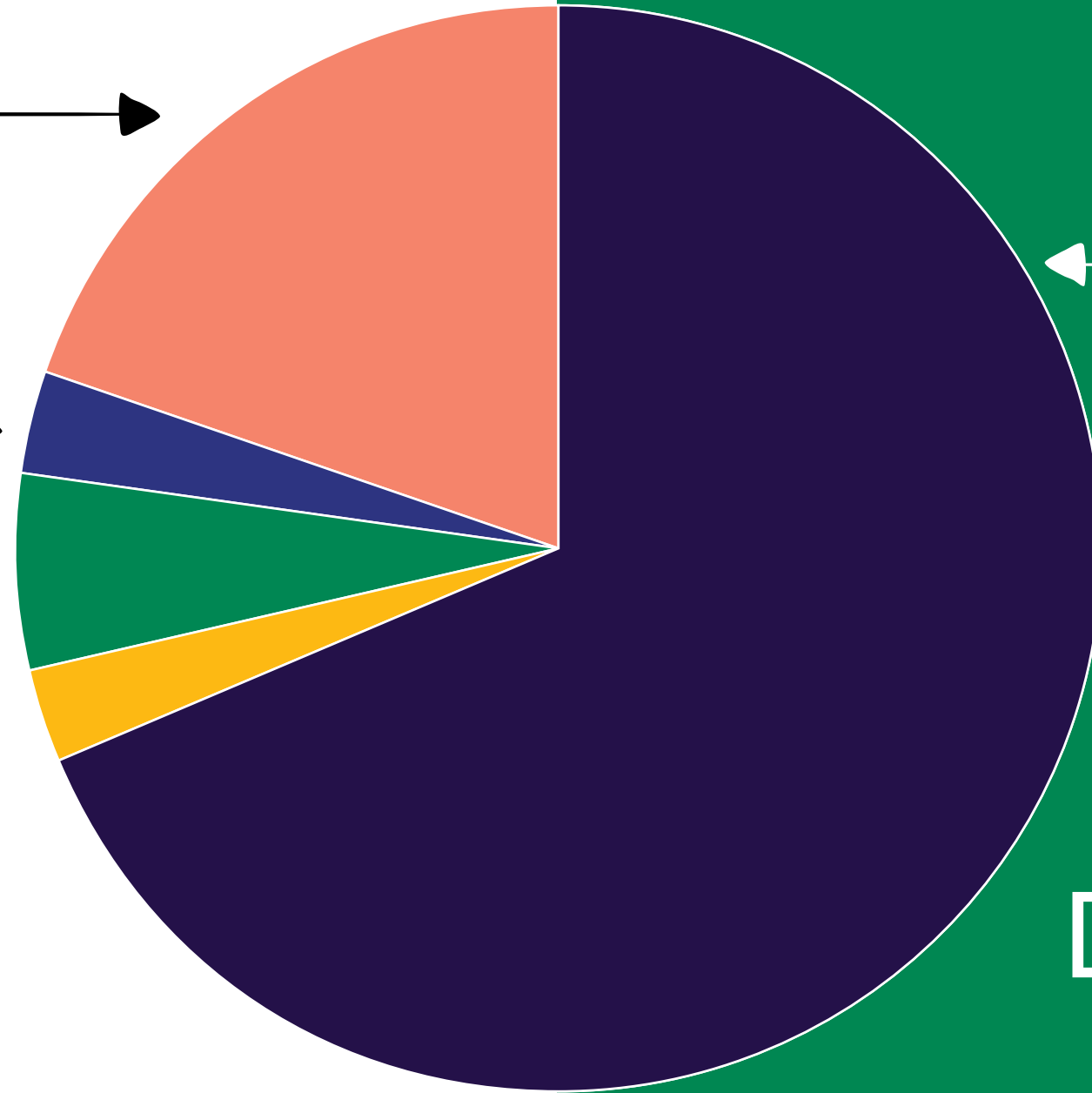


CHEO Foundation
19.47%

Amortization of
Deferred Grants
2.85%

Investment and Other
5.86%

Indirect
Cost Support
3.06%

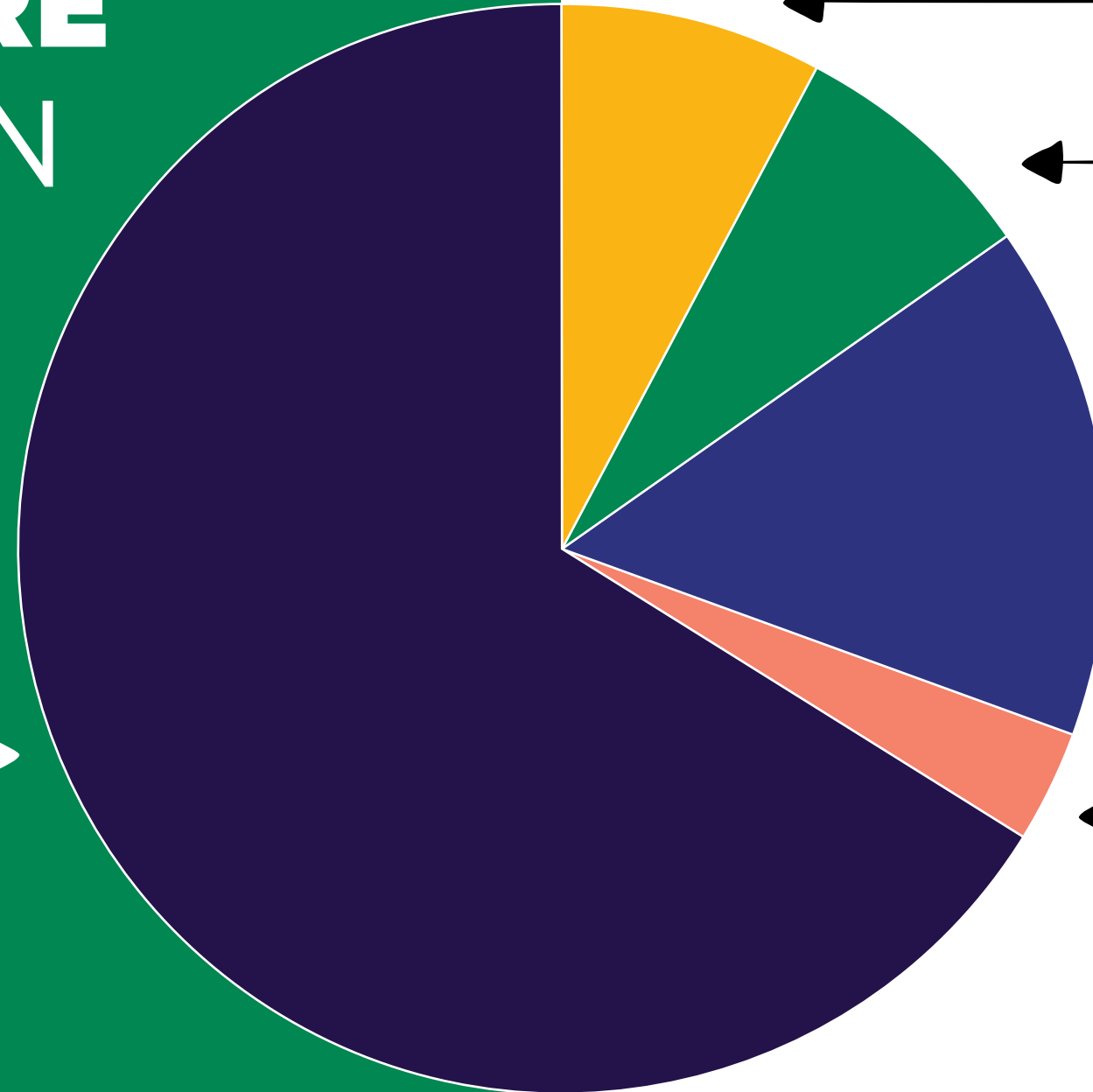


External Grants, Contracts, and
Salary Awards
68.77%

REVENUE DISTRIBUTION 2019-2020

EXPENDITURE DISTRIBUTION 2019-2020

Research and Project Expenses
66.16%

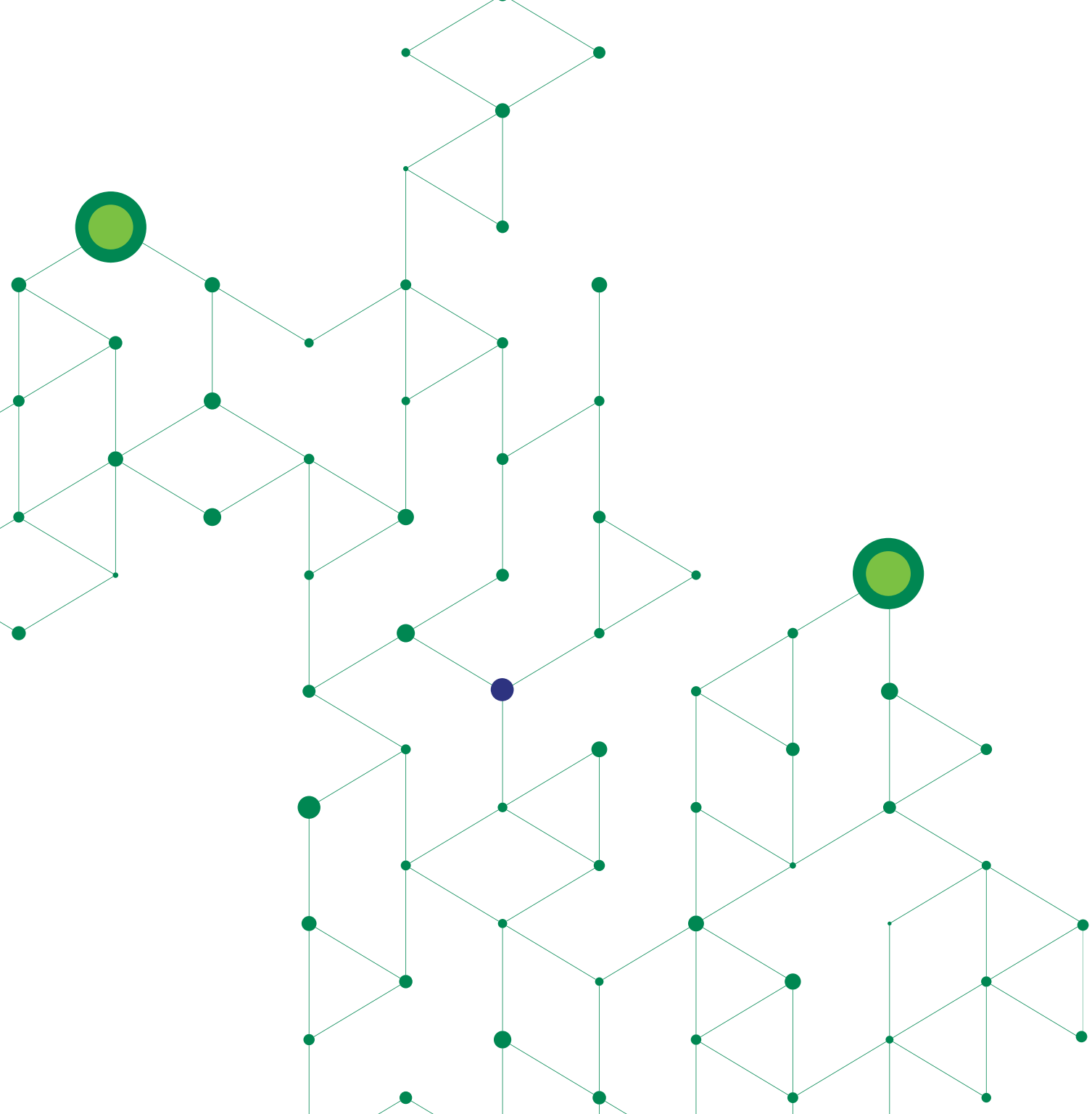


Scientific Salaries
7.88%

Administrative Expenses
7.47%

Other Research Expenses
15.40%

Amortization of Capital Assets
3.09%



SENIOR STAFF

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and Scientific Director

Ms. Rhonda Correll Chief Operating Officer

Mr. Chris St. Germain Chief Financial Officer

Dr. Alex MacKenzie Interim Program Director,
Molecular Biomedicine

Dr. Eric Benchimol Program Director,
Health Information Technologies

Dr. Roger Zemek Program Director,
Evidence to Practice

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