Donor Impact Report: Oncogenomics & PROFYLE

Presented to:

Phoebe Rose Rocks Foundation

CHEO FOUNDATION
FONDATION

September 04, 2019

Mrs. Jenny Doull
Phoebe Rose Rocks Foundation
44 Laval Street
Ottawa, ON K1L 7Z8

Dear Jenny,

We are so thankful for your support of CHEO on behalf of the Phoebe Rose Rocks Foundation. Your generosity is tremendous, but what is behind it makes your gift even more beautiful. The way you have chosen to honour Phoebe is both meaningful and impactful. Thank you! Along with our gratitude, we hope you will be proud of what has been done with your donations. We also hope that you will take pride in knowing that you are helping the children and youth of our community, and beyond, to live their best lives.

As a complement to our recent meeting with Dr. Sawyer, we are attaching a report which provides a snapshot of your gift at work at CHEO. Clinicians and researchers are devoted to finding personalized care for children with rare and difficult-to-treat cancers. Using cutting-edge genome sequencing, that begins at the start of treatment, more can be understood about the biology of the cancer, the risk of recurrence and the potential for more effective alternative treatments for the patient and their families. It is only because of your commitment that our hospital and research staff is able to provide upfront sequencing to families who have no other options.

Thank you for your dedication to making a difference in the lives of the children and youth that CHEO is privileged to serve. The Phoebe Rose Rocks Foundation and the CHEO Foundation share a common mission of supporting children and families affected by cancer. And we are so pleased to be working alongside you! We hope you enjoy learning more about the difference your generosity has made at CHEO over the past year.

With sincere gratitude,

Kevin Keohane
President and CEO
CHEO Foundation
613-737-2782

Pat Zareba
Annual Giving Officer
CHEO Foundation
613-737-2553

PS. Attached please also find a recognition plan for your consideration. We look forward to further discussing an appropriate room naming with you.

CHEO FOUNDATION
FONDATION

Dear Phoebe Rose Rocks Foundation Board Members,

On behalf of Dr. Raveena Ramphal and myself, I wish to express our sincere gratitude for the support that Jenny Doull and the Phoebe Rose Rocks Foundation have provided to us. The funds are being used to investigate patients with rare or unusual tumours, difficult to diagnose tumours and relapsed patients with no further curative treatment options. These are patients who are often not included in mainstream research because of the small numbers involved.

We are happy to share the exciting news that your commitment has recently enabled us to partner with the Terry Fox PROFYLE research program, which as you know is the pan-Canadian collaboration to sequence childhood and young adult cancers. We are very excited to be a part of this large Canadian research consortium which is leading the way and setting new standards in the approach to diagnosis and treatment of pediatric cancer. The funding from the Phoebe Rose Rocks Foundation has allowed us to continue our research, within a national framework, to identify novel drug possibilities and clinical trials which CHEO patients may be eligible for based on the genomic changes identified in their tumours. This is a remarkable program to be a part of, and gives our patients and their families ongoing hope. This is truly an exciting development for pediatric oncology and thanks to your original gift to support the CHEO Oncogenomics program we were well positioned to join Terry Fox PROFYLE as a collaborative site when the opportunity presented itself.

We realize fundraising is hard work and we greatly appreciate all of your efforts. I am looking forward to joining you at the annual Phoebe Rose Rocks Foundation golf tournament, both to share our work and to thank all of the donors in person. We know that there are many worthy causes so we are humbled by your support of our work. We will make sure that every dollar we receive in funding is spent wisely to help this group of patients and their families. Thank you from the bottom of our hearts.

Yours sincerely,

Sarah L. Sawyer

Dr. Sarah Sawyer PhD., MD, FRCPC, FCCMG
Medical Geneticist, CHEO
Associate Professor, University of Ottawa, Department of Pediatrics

R Ramphal

Dr. Raveena Ramphal, MBChB, FRACP, MPH
Pediatric Hematologist Oncologist, CHEO
Associate Professor, Department of Pediatrics, University of Ottawa
Clinical Investigator, CHEO Research Institute

OVERVIEW OF THE PROGRAM:

CHEO sees approximately 10 to 15 patients each year with a difficult to diagnose, refractory, or metastatic cancer. For these children there are no evidence-based therapies, and families struggle with an unknown or poor prognosis. Because the cancers we study are rare, data sharing and collaboration are essential to making progress. To that end, CHEO officially partnered with the Terry Fox PROFYLE Program in the spring of 2019. The scientific aims of PROFYLE are nearly identical to our program, so this is an excellent fit for CHEO patients. This collaboration also allows us to participate in a national database sharing platform and analysis.

CHEO's Oncogenomic research and the Terry Fox PROFYLE program provide personalized care for children with rare and difficult-to-treat cancers. We do this by sequencing the patients' blood to identify genetic risk factors that may have increased their risk of cancer and by tracking genetic changes that are driving their cancer. Some of the 'driver mutations' revealed by this kind of testing have targeted drugs that act against them, which provides alternative and novel treatment options for the patients if the first line drugs haven't worked, or if the patient has a recurrence. For some patients, there is no first line therapy, and sequencing can provide biological insights, more accurate diagnosis, and novel potential treatment options. By targeting the cancer-specific genetic changes, these new therapies also usually have fewer side effects, resulting in a better quality of life. The Phoebe Rose Rocks Foundation and the CHEO Foundation share a common mission of supporting children and their families affected by cancer. **By supporting the CHEO Foundation you are making a state-of-the-art genomics approach available to CHEO patients with cancer through the Oncogenomic research and Terry Fox PROFYLE framework.**

Tumour sequencing is not currently available at CHEO on a clinical basis. **It is through these research programs that patients have access to this upfront sequencing to help guide their treatment plan. Thanks to the support of the Phoebe Rose Rocks Foundation, CHEO is able to continue these very important initiatives directly benefitting several families who otherwise would not have answers to their questions or tailored treatment protocols.** In time, we hope that this approach will be the new standard in pediatric oncology care.

YOUR IMPACT

In summer 2019, after more than a year of discussions, applications and revisions, CHEO received approval from its Research Ethics Board in support of the Terry Fox PROFYLE study. Over the past year we also received renewal approval for the original CHEO Oncogenomic research program. Currently, PROFYLE has funding for 12 non-SickKids patients for the province of Ontario. Given the current number of local pediatric patients who could benefit from this ground-breaking approach there is a huge unmet need. CHEO must be able to fund its own patients. **Your generous contribution will give an additional 5-10 patients from CHEO access to a genomics approach to diagnosis, treatment, and recurrence risks over the following year.**

So far in 2018-19, five patients who were diagnosed with cancer at CHEO have had their tumours and blood sent for sequencing as part of the Oncogenomics research program and three patients were sent as part of the PROFYLE project.

Going forward, the numbers of patients being approved for PROFYLE are expected to be larger than the CHEO Oncogenomics project. We hope to place more CHEO patients who meet the PROFYLE criteria under that program because of its advantageous Canada-wide oncology and biology expertise as well as the informatics pipelines and national data sharing. We already have preliminary data back on some of the patients who are part of the PROFYLE project. The results shed light on recurrence risks for the families and have suggested novel therapies or clinical trials in at least two cases if the standard of care treatment is not successful.

CHEO JOINS TERRY FOX PROFYLE, A NATIONAL RESEARCH PROJECT THAT OFFERS HOPE FOR KIDS WITH RARE AND HARD-TO-TREAT CANCERS.

Terry Fox PROFYLE (PRecision Oncology For Young peopLE) is a multi-institution, pan-Canadian precision medicine research program that aims to change outcomes for children, adolescents and young adults with rare and hard-to-treat cancers. Its goal is to identify and implement treatment recommendations tailored to an individual patient's cancer and better scientific analysis of their tumours.

CHEO is joining this collaboration between more than 30 pediatric cancer research and funding organizations that have come together to give these patients a chance to beat their cancer. Launched by the Terry Fox Research Institute (TFRI) in 2017, Terry Fox PROFYLE enables and encourages data sharing and data analysis by leaders in the field, and knowledge transfer across Canada in a way that will facilitate important advancements for patients. Terry Fox PROFYLE focuses on comprehensive genome sequencing of tumour samples to understand the cancer at a molecular level; vast amounts of data are generated and analyzed at genomics laboratories in Vancouver, Toronto and Montreal, to serve patients in all part of Canada.

Until now, this kind of testing was not always available for young cancer patients especially in rural or remote areas, or cities without a genome centre. This level of collaboration and cooperation among scientists and researchers from across the country to guide diagnosis and treatment is unprecedented. Patients at CHEO, and across Canada, who are eligible for PROFYLE will have access to tumour molecular profiling that can clarify the diagnosis, expand treatment options and hopefully improve prognosis.

Thanks to the Phoebe Rose Rocks Foundation, CHEO is now able to partake in, and benefit from, this program's expertise, data and infrastructure. In addition, CHEO scientists hold leadership positions in the PROFYLE program; most notably, Dr. Jason Berman who co-leads the Tumour Modelling Node whose goal is to develop drug screen methods in real-time for each individual patient's tumour. We expect the results of sequencing tumours to provide more accurate diagnoses and increase novel or targeted treatment options including access to clinical trials for patients who have exhausted conventional treatment options.

Bringing this complex new technology to patients across Canada requires the cooperation of the nation's top researchers and cancer specialists working in research and cancer centres across the country. Together, more than 50 of Canada's top researchers are creating a common platform for tissue biobanking, disease modeling and genomic sequencing that builds on and complements the expertise in hospitals and research facilities throughout Canada. Advances in unraveling the complex genetic changes in individual patients' cancers are allowing us to use this information to identify new targeted

drug therapies and offer new hope to children, adolescents and young adults with relapsed, refractory, or metastatic disease, and their families.

LEADERS IN THE FIELD RIGHT HERE AT CHEO!

CHEO is actively engaged at many levels in the Terry Fox PROFYLE project. In addition to Dr. Jason Berman's role described above, Dr. Sarah Sawyer is the clinical site lead for CHEO's PROFYLE collaboration and as a Medical Geneticist is a member of the Molecular Tumour Board, which meets weekly to review the genomic data and analysis generated from sequencing. She also consents the families and returns part of the sequencing results to the families. Drs. Raveena Ramphal and Lesleigh Abbott are Oncologists who are active in identifying patients with cancer who would benefit from research participation in PROFYLE, and who are responsible to direct treatment changes or alternatives that are advised by the molecular tumour board.

LOOKING FORWARD – PROGRAM OBJECTIVES 2019-2020

- We expect to accrue at least five new patients in 2019-2020 for PROFYLE and will continue with DNA analysis of samples collected in 2019 for both projects. We expect to have one to two patients for the CHEO Oncogenomics study over that timeframe.
- Explore the future sustainability of the PROFYLE project - in a few years we anticipate even more patients enrolling and benefiting from molecular profiling of their tumour, as it will become even more integral to clinical care than it is now.
- We would like to send patient samples to SickKids for sequencing as it is much quicker since many of our patients are also getting treatment there so samples are often already in their pathology department. This change reflects the changing landscape of genomics centres in Canada over the past few years.
- Publishing case reports and working towards larger publications with the PROFYLE team to share what we have learned with others, and to recognize donors who support this project.
- Studying the biology and tumour progression of novel cancer predisposition genes through the model organisms network.

THANK YOU!

With the Phoebe Rose Rocks Foundation's continued support, we are positively impacting the lives of children and youth at CHEO and across Canada faced with hard-to-treat cancers by increasing our ability to offer genomic sequencing and accelerating our ability to offer them access to new therapies. Thank you for your continued generosity and thoughtfulness!