



THE TRACE PROGRAM

IMPACT REPORT 2019





Taylin and Colton with Buddy the Miner



MINING FOR MIRACLES

SUPPORTING CHILD HEALTH FOR OVER 30 YEARS

The mining industry's commitment to BC Children's Hospital (BC Children's) began in 1988, making it one of BC Children's longest standing supporters. And because of that incredible and steadfast dedication over the past 30 plus years, volunteers from across the mining industry have raised more than \$30 million.

Reaching this incredible milestone was possible because of the generous contributions from individuals and companies who support the Mining for Miracles campaign year after year through fundraising initiatives such as the Pie Throw, Hooked on Miracles, Diamond Draw and the online auction. These events are successful because of the tireless efforts of event organizers, volunteers and participants. Thank you for your commitment to helping kids throughout the province.

The cutting-edge projects you carefully selected to rally behind have yielded tremendous benefits for the kids of BC. Through support of facilities, innovative programs and specialized medical equipment at the Hospital and BC Children's Research Institute (Research Institute), Mining for Miracles is helping to keep BC Children's at the forefront of pediatric care excellence.

“We are grateful for this gift which has allowed us to set up new technologies that will improve the lives of kids in BC who have had a transplant.”

– Dr. Megan Levings, lead, childhood diseases research

In November 2018, Mining for Miracles was celebrated for their outstanding fundraising efforts as they completed their pledge to support the establishment of the Transplantation and Cell Therapy (TRACE) Program. The TRACE program will provide children across British Columbia with personalized medicine using the patient’s own cells to prevent rejection of organ transplants, kill cancer cells and fight infection. This program is positioned to develop and provide hundreds of children and families with expanded treatment options. Your fundraising efforts are ushering in a new era of care for BC’s kids.

Thank you for helping kids shine.



Mining for Miracles November 2018 celebration



THE TRACE PROGRAM

Hematopoietic stem cell transplant (HSCT), is a procedure where cells from the bone marrow are transplanted into a patient to create new blood cells. HSCTs were a major medical advancement when introduced in the second half of the 20th century and are the only established cell-based immune therapies to treat blood cancers such as leukemia. As groundbreaking as transplantation is, it unfortunately comes with a host of potential issues including rejection, infection, and other life-long health issues. Today, medicine is on the precipice of an amazing revolution as patients benefit from research aimed at tailoring transplantation and cell therapies to their individual needs.

The TRACE Program is at the intersection of clinical care and research. Ultimately, it will allow BC kids to access novel cell and transplantation therapies while enabling researchers to further discover and implement new therapies in the future.

Technical advances in the ability to isolate, genetically manipulate and grow various cell types outside the body mean scientists can design targeted cell therapies to fix specific problems. For example, scientists can isolate a patient's own immune cells, modify them in the lab to redirect them to a specific task (such as attack cancer cells), then return these cells to the patient to carry out the task.

The transplantation field is rapidly becoming very targeted and personalized in its approach. Mining for Miracles' support in establishing the TRACE Program has allowed BC Children's to be ideally positioned to move a variety of novel research approaches from exciting findings in the lab to a clinical setting—ultimately for the benefit of children and their families.

GOALS

EXPAND TREATMENT
OPTIONS FOR HUNDREDS OF BC KIDS

ENABLE RESEARCHERS TO
DISCOVER AND DEVELOP
NEW CUTTING EDGE THERAPIES
NOT PREVIOUSLY AVAILABLE
TO CHILDREN RECEIVING A
TRANSPLANT

ALLOW PHYSICIANS TO **BETTER
PREDICT** WHEN A CHILD IS
AT RISK OF DEVELOPING
INFECTION, LATE COMPLICATIONS
OR TRANSPLANT REJECTION

USE **INNOVATIVE
METHODS** TO
MONITOR PATIENTS
UNDERGOING CELL
THERAPY AND
TRANSPLANTATION,
**IMPROVING
THEIR
CLINICAL CARE**

ENABLE CHILDREN
AND FAMILIES IN BC
TO **ACCESS**
THE BEST POSSIBLE
CELL THERAPIES

ACHIEVEMENTS

Mining for Miracles' generous contribution of \$2.9 million helped enable the establishment of two core facilities for the TRACE Program: the clinical cell therapy and translational biomarker facilities, which are essential in order to bring novel cell therapies and diagnostics to BC Children's.

Clinical cell therapy facility

The clinical cell therapy facility is a significant step in personalized medicine, allowing BC Children's physicians to isolate a patient's cells and modify them to do a specific task. This will transform patient care by tailoring therapies to the needs of each individual patient. Killing cancer cells, stopping viral infections, offering new possibilities to children with incurable genetic diseases and preventing rejection after transplantation are a few examples of the incredible ways in which cell therapy can be utilized to expand the treatment options for hundreds of BC Children.

Translational biomarker facility

The first of its kind in Canada, the translational biomarker facility is dedicated to the innovative monitoring of patients undergoing transplantation and cell therapy. This monitoring aims to assist physicians in personalizing therapies to help kids showing early warning signs of transplant rejection. As an example, it is hoped that in the near future, rather than risky or painful biopsies to test for possible rejection, a simple urine test will be a first line of surveillance to understand if the transplanted organ is at risk of being rejected. Using this personalized approach, the TRACE team hopes to minimize the risks of transplant.

These achievements have enabled the TRACE Program to:

- > Help 250 children living with organ or stem cell transplants in BC and position BC Children's as a world leader in innovative blood screening tests to reduce risks associated with transplant
- > Provide a better and safer HSCT for children in BC with leukemia or incurable genetic diseases
- > Improve the lives of families by allowing for personalized approaches to transplantation that are safer and mean less time spent in hospital
- > Position BC Children's as a leader in clinical cell therapies, transplantation and research into new treatments for cancer and diabetes

LOOKING AHEAD

Thanks to Mining for Miracles' support in establishing the TRACE Program, the Research Institute can now focus on conducting innovative research projects. These projects will test biomarker applications and cell therapies and enhance precision analysis with a goal of expanding and improving therapeutic opportunities. The team also intends to transition some of the research approaches into standards of care so that in the future they are available to any child in BC who needs them.



State-of-the-art equipment allows dangerous cells to be removed before a stem cell transplant.



MEET THE TRACE MANAGEMENT TEAM

DR. TOM BLYDT-HANSEN

Associate Professor of Pediatrics

Director, Multi Organ Transplant Program

Areas of expertise:

- > Clinical and translational research in solid organ transplantation
- > Biomarkers of transplant injury and rejection



DR. SOREN GANTT

Associate Professor of Pediatrics

Head, Division of Infectious Diseases, UBC

Director of Clinical Research

Area of expertise:

- > Infectious diseases
- > Viral infections in immunocompromised patients
- > Clinical and translational studies of pathogenesis





DR. MEGAN LEVINGS

Professor, Department of Surgery

Lead, Childhood Diseases Research

Areas of expertise:

- > Regulatory immune cell therapy
- > Immune regulation in transplantation and autoimmunity



DR. KIRK SCHULTZ

Professor of Pediatrics

Director, Michael Cuccione Childhood Cancer Research Program

Areas of expertise:

- > Clinical studies in HSCT and cellular therapeutics
- > Immune treatments of leukemia
- > Chronic graft-versus-host disease

DR. CARON STRAHLENDORF

Division Head, Pediatric Hematology/Oncology/BMT

Areas of expertise:

- > Clinical trials
- > Apheresis
- > Bioethics



DR. SUZANNE VERCAUTEREN

*Head, Department of Pathology
and Laboratory Medicine
Director, Cellular Therapy and
Flow Cytometry Laboratories*

Areas of expertise:

- > Cellular therapy
- > Flow cytometry
- > Biobanking







RESEARCH PROJECTS IN TRACE

Mining for Miracles support in establishing core facilities for the TRACE Program has allowed the program to leverage this gift to secure additional funding that is enabling innovative research. BC Children's is fortunate to have some of the world's leading cell therapy and transplantation researchers at the helm. Here are a few examples of the work Mining for Miracles has made possible:

RESEARCHER SPOTLIGHT

DR. TOM BLYDT-HANSEN – KIDNEY TRANSPLANT REJECTION URINE BIOMARKER

Kidney transplantation can be a life-saving treatment for children with kidney disease. However a major threat to success is rejection, where the body's immune system attacks and destroys the transplanted kidney. If rejection isn't treated early, the results can be devastating.

Currently, tests that monitor kidney function can miss early or mild rejection, and repeated kidney biopsies are risky and unpleasant. Dr. Blydt-Hansen's team is working to develop techniques to identify signs of kidney injury and rejection in children who have had a kidney transplant using a simple urine test.

Metabolites (small molecules produced by cells) and chemokines (molecules that signal inflammation) are known to change when rejection occurs. The research compares changes in samples taken over time to detect rejection early. This work will reduce the number of biopsies and improve the health and long-term kidney function of children with a transplant.

The team has tested a variety of samples to confirm the accuracy of this method to predict kidney transplant rejection. With the protocols in place to make the collection storage of samples clinically viable, BC Children's is poised to soon make this process a routine clinical test for children with kidney transplants.

"This research has the potential to help children by enabling early detection of rejection and by tailoring treatment and monitoring to each child's individual needs. This will improve the quality of life for children who have had kidney transplants."

RESEARCHER SPOTLIGHT

DR. SUZANNE VERCAUTEREN – PURIFYING CELLS FOR TRANSPLANT

The cellular therapy laboratory, led by Dr. Vercauteren, is filled with new, state-of-the-art equipment thanks to the incredible support of Mining for Miracles. One of the functions of this lab is to prepare hematopoietic stem cells (HSCs) for transplantation.

Immune cells present in HSCs can increase the risk of complications of HSCTs. The new infrastructure now in place allows HSCs to be purified or to remove specific types of undesirable immune cells. With these modifications, it may soon be safer for a child to receive a transplant from one of their parents or siblings and the risk of complications from the procedure are reduced.

“With this state-of-the-art facility we finally can treat children from BC closer to home so they don’t need to travel out of the province or even the country to receive treatment.”

The new infrastructure also allows the isolation of certain immune cells that are able to kill off virally infected cells and then infuse them into children who are not able to control a viral infection because their own immune system is not working properly. It is hoped that this new method will enable a clinical trial of children afflicted with a severe infection after an organ or bone marrow transplant. In the future, this equipment could also help to produce immune cells that can attack cancer cells.



Kendra, age 13 & mom

RESEARCHER SPOTLIGHT

DR. KIRK SCHULTZ – GRAFT-VERSUS-HOST DISEASE

Dr. Schultz and his team focus on conducting research that tests cell therapies for children with cancer and enhances precision analysis of patients in order to expand and improve therapeutic decisions. One of the novel research projects focuses on the creation of biomarker tests to revolutionize HSCTs.

Stem cell transplants are the most common immune therapy used to date. Even with newer therapies such as the genetically-targeted CAR T-cell therapy, where cells are modified to perform a specific task such as killing cancer cells, transplants are often needed to ensure a lifelong cure. A particularly debilitating condition that can afflict individuals who have undergone a blood and marrow transplant is graft-versus-host disease (GvHD), where the donated cells view the recipient's body as foreign and attack, sometimes causing life-long disability. This is spurring on an innovative research project to examine the late occurring forms of GvHD, which currently affect approximately 50 per cent of children receiving an HSCT.

Dr. Schultz's team is pursuing the creation of biomarkers that could result in the ability to personalize blood and marrow transplants to make immune therapies safer, prevent complications, lower the risk of rejection and limit the development of GvHD. This will help not only children in BC, but also kids and adults worldwide.

“With Mining for Miracles’ support of the TRACE Program and development of the TAB lab, I can now proudly say that BC Children’s is leading efforts worldwide to develop personalized approaches to safer and better HSCTs.”

The current work partially supported by the TRACE Program includes the development of the transplantation applied biomarker laboratory (TAB lab). The TAB lab, led by Dr. Schultz, has positioned itself as the premier research group worldwide leading the development of tests that will allow personalized monitoring of children and adolescents living with stem cell transplants.





PATIENT SPOTLIGHT JEREMY'S STORY

The Monday after Mother's Day in 2015 started out like any other for Jeremy. At his family's dairy farm in Cobble Hill, then 8-month-old Jeremy had a nap like he did most days. However, on this day he woke with a large lump on his side.

Michelle, Jeremy's mom, immediately took him to the emergency room at Cowichan District Hospital in nearby Duncan. An ultrasound revealed it was a tumour, and Jeremy was taken to BC Children's.

"No parent wants to hear their child has a tumour," says Michelle. "We didn't know what to think. We just had to take it day by day."

At BC Children's Jeremy had surgery to remove his right kidney along with the tumour, which was a Wilms tumour—a type of childhood kidney cancer. Doctors suspected Denys-Drash syndrome, a rare kidney disorder that can lead to kidney failure in young children. Ninety per cent of children with Denys-Drash syndrome develop Wilms tumour. After five months of chemotherapy, the diagnosis was confirmed in September.



A year after his first surgery, Jeremy began receiving dialysis at home to remove waste and extra chemicals from his blood. He would spend 12 hours each day hooked up to the dialysis machine. The Denys-Drash diagnosis meant that his other kidney was at risk of developing a Wilms tumour, and Jeremy needed to be cancer-free in order to have a transplant. His left kidney was removed in May of 2017.

Two days after his third birthday that September, Jeremy received the best present anyone could hope for—a kidney donated by his father, Jason. The surgery was not without complications, though. Jeremy developed a clot in the transplanted kidney, requiring an additional surgery immediately after the transplant. Jeremy spent a full week in intensive care and received dialysis for two weeks until the new kidney began working on its own.

Jeremy's parents are grateful for all of the staff at BC Children's, and especially Dr. Tom Blydt-Hansen, who continues to follow Jeremy. "As a parent going through this, you don't hear everything being said to you," says Michelle. "It goes in one ear and out the other. Everyone did such a good job explaining things over and over again until we understood it, and answering our endless questions."

Today, Jeremy is a happy 4-year-old boy. He continues to see Dr. Blydt-Hansen every two months and is a part of a two research studies, including the urine biomarker study that hopes to someday eliminate the need for biopsies to test for possible rejection. Jeremy is too young to understand what a remarkable journey he has been on and dislikes being poked by needles, but being off dialysis has given him a lot of freedom. He loves being outside with animals on the farm, especially the baby cows.

Jeremy's family needs to continue monitoring his transplanted kidney, and he needs to take anti-rejection medications. He experienced a mild rejection last year requiring steroids, which Dr. Blydt-Hansen helped to put into perspective. "He described it as being like a campfire," says Michelle. "If you leave it unattended, it can become a large wild fire but if you extinguish it right away, rejection is easier to control."





THANK YOU MINING FOR MIRACLES

We are so grateful to the mining community for supporting BC Children's Hospital. Mining for Miracles' continued support helps turn novel ideas and research into world-class care.

Thank you for working so hard for innovation in research, and for helping BC Children's remain a centre of excellence in health care for kids and families.

We dream of a day when children don't get cancer, a day when there are no complications from a lifesaving kidney transplant. When it comes to our vision that every child is healthy, we know there will always be work to do. In spite of the marathon ahead of us, we are sprinting to the next hurdle. And with your help, we are going to clear it.

Together, we are built to heal.



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