

Press release
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A Montreal research team headed by the eminent Janusz Rak, Ph.D. leads the way to the 'next frontier' in pediatric cancer screening, diagnosis, and treatment

Exciting scientific advances in pediatric liquid biopsy research are fueled by a \$5 million donation from the Fondation Charles-Bruneau and \$1 million from the CIBC Foundation

Montréal: January 12, 2023 – Great news ahead for the screening, diagnosis, and treatment of solid tumours pediatric cancers. A distinguished team of scientists with the Child Health and Human Development Program at the Research Institute of the McGill University Health Centre (RI-MUHC) is developing a simple blood or urine test - a liquid biopsy - that will replace risky surgical biopsies to determine if a suspicious lump is cancerous and pinpoint the exact type of cancer.

The Liquid Biopsy Research Project is led by scientist Janusz Rak, Ph.D., a world leader in nanotechnology research on novel biomarkers (the use of technology to recognize molecules associated with cancer). His research increases global knowledge of liquid biopsies, which show great promise for improving the diagnosis, treatment, and monitoring of solid cancerous tumours, including brain cancers, gliomas, medulloblastomas, and bone tumours, such as sarcomas. These are the deadliest forms of pediatric cancers, with a mortality rate of 20-40% in children and adolescents with these cancers.

In Canada, cancer remains the number one cause of death by disease in children, and brain cancer is the deadliest pediatric cancer. Every year, an average of 943 children are diagnosed with the potentially fatal disease, and 65% of these children will have a solid tumour, such as a brain tumour or a sarcoma (cancer of the bone or connective tissues).

For several years, the research community has known cancerous tumours shed small amounts of material such as cells, DNA, RNA, and proteins into the blood and other bodily fluids. This knowledge is rapidly improving the landscape of adult oncology but, unfortunately has yet to have any impact thus far on our understanding of childhood cancers.

There is a glimmer of hope on the horizon. It turns out that cancer cells release billions of small "bubble-like" structures called extracellular vesicles or exosomes, which are like miniature replicas of cancer cells. If exosomes are captured, they can reveal what type of cancer lurks

in the body, what type of malignant cells the tumour contains, and possibly how to attack them with treatment. Recent advances in technology and bioinformatics have improved the capture and analysis of these exosomes to diagnose and monitor the behaviour of cancerous tumours paving the way for liquid biopsy tests in children.

The traditional way to diagnose cancer

Usually, to determine if a child has a cancerous tumour, surgeons perform a surgical biopsy removing a small amount of tissue with a needle or, in some cases, cutting out an entire lump or nodule. But biopsies are invasive, and there are risks of multiple complications. In the case of some brain tumours, the location of the tumour makes doing a biopsy impossible, as it would put the child's life at greater risk. If cancer metastasizes (spreads to other body parts), numerous tumours make diagnosing even harder.

Liquid biopsies are a game changer

The story of exosomes and liquid biopsies started about a decade ago. At the time, researchers knew cancer cells became malignant because genes go awry and make cells divide at an abnormally fast pace, causing tumours. To treat cancer, doctors need to know which genes are affected, as drugs now target specific cancer genes. But how do you know which genes are involved when the tumour's location prevents a biopsy?

Janusz Rak was the first researcher in the world to discover that genes responsible for solid cancer tumours can be detected if they are in exosomes released into the bloodstream – **this is one of the most important scientific discoveries concerning cancer exosomes**. These genes can then be monitored and identified with a liquid biopsy to guide diagnosis and treatment.

"Liquid biopsies will open up new perspectives in the treatment of childhood cancers," explained Janusz Rak, Ph.D., Senior Scientist, Child Health and Human Development Program, RI-MUHC. "They are virtually pain-free and risk-free. Plus, they would allow us to repeat diagnostic tests to ensure the child's treatment is working and, if not, to switch to a different medication immediately. We would also eventually be able to screen at-risk children for cancer before an official diagnosis, which would allow the medical team to catch the disease earlier. With most cancers, an earlier diagnosis improves a child's chance of survival. Finally, by thoroughly understanding tumour biology, we could create personalized treatments for each child."

"By investing in the best researchers and cutting-edge research, Fondation Charles-Bruneau has played a crucial role in the fight to find a cure for pediatric cancers," says Rébecca Dumont, executive director of Fondation Charles-Bruneau. "Over the past 30 years, the cure rate has increased from 30% to 80% for all pediatric cancers. This is tremendous progress, but there is still a lot to do. That's why we are proud to have awarded this \$5 million donation to The Montreal Children's Hospital Foundation to advance the groundbreaking research of Janusz Rak and his team that will open the door to liquid biopsies to improve the outcomes for children with solid tumours."

Janusz Rak has surrounded himself with experienced researchers who could bring new insights into other forms of cancer, such as neuroblastoma, Wilms' tumour, retinoblastoma, non-Hodgkin's lymphoma, and others.

The key collaborators are:

- Dr. Nada Jabado, a researcher in the Child Health and Human Development Program at the RI-MUHC and oncologist at The Children's, is internationally recognized for her work in pediatric cancer genomics.
- Dr. Claudia Kleinman a Data Scientist who knows how to make sense of molecular puzzles. Data scientists extract valuable insights from biological information and develop and use mathematical models for statistical analysis.
- Dr. Claudia Kleinman bioinformatician who knows how to make sense of molecular puzzles. Bioinformaticians create and maintain databases of biological information and develop and use mathematical models for statistical analysis.
- Livia Garzia, Ph.D., researcher in the Cancer Research Program at the RI-MUHC and specialist in sarcoma and medulloblastoma (brain tumour) research.
- Dr. Joanna Przybyl, an investigator and liquid biopsy expert in the Cancer Research Program at the RI-MUHC. She is studying liquid sarcoma biopsies.
- Sarah Mahshid, Ph.D., Assistant Professor in the Department of Bioengineering at McGill University and an expert in nanotechnology and creator of new methods for exosome analysis.

"The CIBC Foundation is proud to support the talented team at The Montreal Children's Hospital (The Children's) and the Child Health and Human Development Program at the RI-MUHC, and its mission of creating a healthier future for children with cancer through this groundbreaking development," said Mme Rosa Trunzo, Senior Vice-President and Region Head, Quebec region, Personal and Business Banking, CIBC. "Supporting causes that are important to our clients and communities is integral to the CIBC Foundation and brings us closer to realizing our collective ambition of creating a future where no one has to fear a cancer diagnosis."

Stronger together

"The Children's Foundation is very grateful to its visionary partners, the Fondation Charles-Bruneau for its \$5 million donation and the CIBC Foundation for its \$1 million donation to support the cutting-edge research of Janusz Rak and his team. This research will make an enormous difference in the lives of children with solid tumour cancers, both here in Quebec and around the world," says Renée Vézina, President of The Montreal Children's Hospital Foundation. "Thank you to our donors for helping The Children's find *Unexpected Ways to Heal*."

Liquid Biopsy Research Project is one of many transformational projects made possible thanks to The Children's Foundation's *Unexpected Ways to Heal* campaign, which aims to raise \$200 million by December 2026. It is the most ambitious fundraising objective for a pediatric hospital in Quebec's history.

ABOUT THE MONTREAL CHILDREN'S HOSPITAL FOUNDATION

The mission of the Foundation is to inspire and mobilize the community to support innovation in clinical care, research and teaching at The Montreal Children's Hospital. The Children's is the pediatric teaching hospital of McGill's Faculty of Medicine and Health Sciences. It supports the Child Health and Human Development Program of the Research Institute of the McGill University Health Centre, which, in 2022, ranked among the top three research institutes in Canada. The Foundation has launched a major campaign with a goal of \$200 million, the most ambitious fundraising objective for a pediatric hospital in Quebec's history. Funds raised will advance groundbreaking projects that will push the envelope in pediatrics on an international scale and help the Children find *Unexpected Ways to Heal*. Since its inception in 1973, the Foundation has raised more than \$580 million, which has helped sick children in Montreal and around the world get back on their feet and return to their mischievous ways. Because a bratty kid is a healthy kid, for more information, please visit childrenfoundation.com.

For more information, please contact:

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Download photos of Brandon, the little boy diagnosed with cancer, here:

<https://www.dropbox.com/sh/ynnradbojwcquap/AABKWQbkkr10XVFDj21RbjoVa?dl=0>