



## Annual Report 2017 - 2018



# The OIRM investment

The Ontario Institute for Regenerative Medicine (OIRM) continues to showcase extensive growth. In just three years, over half of the organization's Disease Team technologies have shifted from mid-pipeline to pre-clinical and early stage clinical trials.

OIRM funded twelve projects for 2017-18 in four Ontario cities with a total investment of just over \$3.5 million.

**5 Disease Team projects**  
**7 New Ideas projects**  
**2 post-doctoral fellowships**

Not only is the organization growing and expanding, bringing on an additional 41 new members in 2017-2018, the technologies are evolving through the pipeline and getting closer to commercialization and the clinic.

# Company creation and commercialization of OIRM-generated technologies

The research OIRM is supporting is having significant impact in the province and around the world.



A company co-founded by Dr. Duncan Stewart recently launched a multi-centre clinical trial across nine sites in Canada, utilizing a cell

and gene therapy that was developed in Ontario to treat pulmonary hypertension. Northern Therapeutics, an Ontario-based company is leading this development, in partnership with United Therapeutics, a multi-billion market cap US-based company. Cells for this trial will be manufactured in Ontario at the Ottawa Hospital Research Institute, bringing additional funds into the province and highlighting the Ontario-first mandate of OIRM.



Dr. Michael Rudnicki, an OIRM-funded researcher and board member has recently founded Satellos Bioscience Inc., a new entrant on Canada's biotechnology scene.

This is a collaboration with Bloom Burton and Frank Gleeson, who now serves as the President and CEO of Satellos.

Satellos uses Dr. Rudnicki's landmark discoveries around muscle stem cell division and differentiation and harnesses these mechanisms using small molecules to create a new therapeutic paradigm. This treatment has shown great potential in the area of Duchenne Muscular Dystrophy, a severe genetic disease of children that currently leads to death early in adulthood. Satellos recently closed a seed round of financing and is yet another example of Ontario innovation in the healthcare and economic development space.



OIRM continues to support direct research costs for Dr. Michael Laflamme (University Hospital Network), who is developing

the lead technology for BlueRock Therapeutics. OIRM has invested over \$2.5M since 2014, which supported the technical advances that, in part, secured a \$225M investment from Bayer and Versant Ventures to develop BlueRock Therapeutics. BlueRock Therapeutics now has 30 employees in Ontario and continues to expand.

**Read more about the work of Michael Laflamme on our website!**

These are just three key examples of OIRM's ability to identify and support high capacity research with high commercialization potential.

OIRM is also actively working with venture capital (Versant Ventures), investment banking (Bloom Burton), industry (BlueRock, Satellos and Northern Therapeutics) and CCRM to support investment in new technologies.

# Clinical trials: moving towards uptake in the global marketplace

Cell therapy clinical trials are essential for evaluating the safety and efficacy of a proposed treatment. They are also a critical component to ensure the uptake of Ontario-generated technologies in global markets and for attracting commercial investment.

OIRM's support has increased the number of cell and gene therapy trials in Ontario from eight in 2016 to 11 in 2018. Six of these trials are multi-centre across Canada with some expanding into the United States through partnerships with highly-regarded academic centres. Once therapies are deemed safe and effective via a clinical trial, it ensures rapid uptake of the technology in the marketplace.



In 2017, the results of the world's first clinical trial for the treatment of septic shock with mesenchymal stem (stromal) cells were published in the *American Journal of Respiratory and Critical Care Medicine*. Led by OIRM clinician-researcher, Dr. Lauralyn McIntyre, senior scientist and physician at the Ottawa Hospital, this Phase I trial examined the safety of the cells in 30 patients (9 intervention; 21 control). OIRM is now supporting efforts to expand this technology into a larger, multi-centre Phase II clinical trial across Canada with plans to expand this into an international trial. Despite decades of

research examining different therapies, none has proven successful and supportive care remains the mainstay of therapy, at a cost of approximately \$4-billion in Canada annually.



Dr. Bernard Thebaud of the Ottawa Hospital is also close to clinical trial and the commercialization of his stem cell therapy to

support lung development in premature babies. OIRM funds have been critical in moving his project forward and towards the clinic. Extreme prematurity is the main cause of mortality and morbidity in children before five years of age. The most severe complication is bronchopulmonary dysplasia (BPD), a chronic lung disease that follows ventilator and oxygen treatment for acute failure to breathe. BPD also adversely affects the brain and leads to blindness. Currently, there is no treatment for BPD. Because these injuries occur in developing organs, consequences are life-long and carry a high economic burden. Thus, effective interventions at this stage of life provide exceptional value.

“We’re on the verge of bringing what we find in the lab to patients. There’s a grey zone where it’s difficult to get funding when we have a promising cell product that we want to get to clinical trials and OIRM has bridged a gap there and filled an unmet need. We hope that in the next three years, we will be able to show that this was a good return on investment by initiating a first-in-human trial in babies to protect their lungs.” Dr. Bernard Thebaud

**Read more about Dr. Thebaud’s work on the OIRM website.**

Dr. Duncan Stewart’s SAPPHERE trial, a Phase 2 study to establish the efficacy of a gene-enhanced cell therapy for pulmonary hypertension, is currently taking place at multiple sites across Canada. OIRM is working with Dr. Stewart to develop a partnership with the California Institute for Regenerative Medicine to undertake a sister trial in the US, together with a network of California-based institutions.

In 2018-2019, the Institute expects to see at least two more early phase clinical

trials started with OIRM as a key driver in funding and stakeholder engagement. OIRM's portfolio includes investments in both early stage trials and larger multi-centre trials to ensure that new ideas and highly established ones continue to develop along the technology pipeline.

To help increase the speed of uptake for Ontario technologies in the global market, OIRM has appointed two Regulatory and Manufacturing Advisors - Drs. David Courtman (OHRI) and Sowmya

Viswanathan (UHN) - who have a wealth of expertise in implementing cell therapy trials to provide consultations to OIRM Disease Teams as they move towards the clinic.

OIRM is also an ongoing participant in the Health Canada Cell Therapy Stakeholder Group, acting as a conduit for providing key regulatory information and strategies to its investigators as they move their cell therapies into clinical trials. This allows for continued collaboration and

understanding as Ontario's investigators take steps to move their technologies to patients.

In addition, OIRM is collaborating with the Translational Research Program at the University of Toronto to develop a scorecard to evaluate the translational scope and capacity of its Disease Teams and to provide investigators with a guided approach to a successful transition to early phase clinical trials.

## CELL THERAPY CLINICAL TRIALS IN ONTARIO 2018-2019

### BRAIN AND/OR NERVES

**Phase I** using a diabetes drug for brain repair in children with Multiple Sclerosis: Toronto

**Phase II** using stem cells for Multiple Sclerosis: Ottawa

**Phase II** using a diabetes drug for brain repair in children with Cerebral Palsy: Toronto

**Phase III** using a diabetes drug to stimulate brain repair for malignant brain tumours: Toronto

### GENETIC DISORDERS

**Phase I** using genetically modified stem cells for Fabry Disease: Toronto

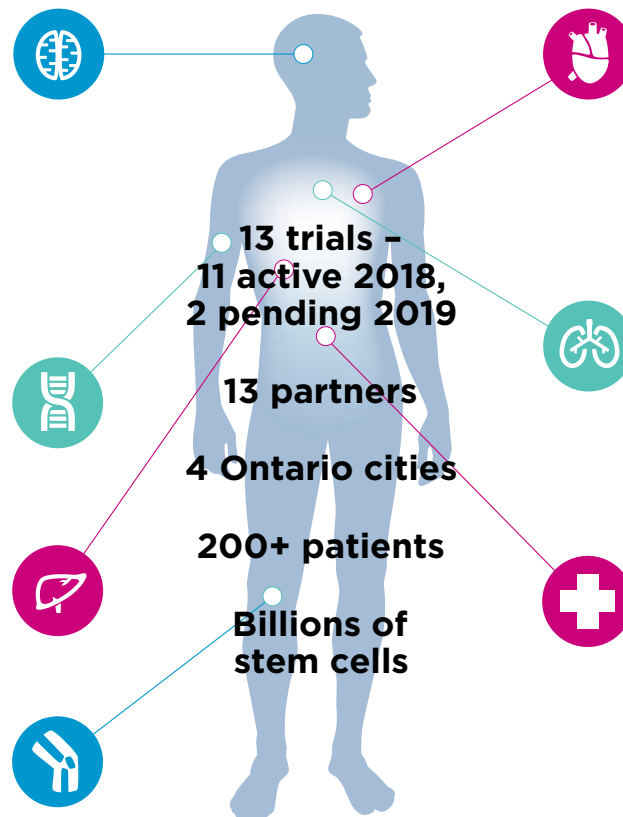
### LIVER

**Phase II-III** using stem cells to induce tolerance in recipients of liver transplants: Ottawa and Toronto

### JOINTS

**Phase I-II** using stem cells for knee osteoarthritis: Toronto

**Phase III** using a cartilage tissue implant grown from the patient's own cells: London



### HEART

**Phase II** using genetically modified stem cells following a heart attack: Ottawa and Toronto

**Phase II** using stem cells for advanced heart failure with left ventricular assist device: Toronto

### LUNG

**Phase I** using stem cells for bronchopulmonary dysplasia in pre-term infants. Ottawa (Pending 2019)

**Phase II** using genetically modified stem cells for pulmonary arterial hypertension: London, Ottawa, Toronto and Hamilton

### CRITICAL CARE

**Phase II** using stem cells for septic shock: Hamilton, Ottawa, Toronto (Pending 2019)

All trials lead by OIRM investigators

All trials sponsored/funded by some or all of the following: UHN, Ottawa Hospital Research Institute, SickKids, Dr. Gary Levy, Dr. Jas Chahal, Histogenics, CIHR, Stem Cell Network, Ozmosis Research Inc., Dr. Annetine Gelijns, National Heart, Lung and Blood Institute, Northern Therapeutics and OIRM



# International partnerships boost Ontario's economy

OIRM is a global competitor in the area of cell therapy clinical trials and has had continued success in developing top level partnerships from the US, the UK and the EU to support global clinical trials of Ontario-generated technologies.

The California Institute for Regenerative Medicine (CIRM) is a key partner for OIRM. CIRM's revised programming even allows for funds to flow into Ontario in support of international trials. OIRM is in discussions with CIRM about jointly supporting two clinical trials of Ontario-generated technologies in both California and Ontario. If the trials are successful, this will pave the way for access to the American market and facilitate broad

uptake of these technologies. International clinical trials evaluate safety and efficacy and create global business opportunities.


Many of these jurisdictions have significant funds available for cross-border partnerships, which can lead to economic benefits for the province.

OIRM continues to develop international relationships to increase Ontario's leverage in the global market. These partnerships

confirm Ontario's highly-sought expertise in the regenerative medicine space.

In 2017, OIRM attended the International Society for Cellular Therapies event in London, UK, as part of a Canadian pavilion to represent the interests of Ontario researchers in this field and was present at multiple international stem cell conferences and events to ensure Ontario's voice was at the table.

# OIRM technologies poised to reduce healthcare costs



Saving healthcare dollars is critical to Ontario's economy and OIRM is pleased to be supporting several key Ontario-based programs that are poised to generate savings. Stem cells offer the opportunity to not only treat disease but to provide curative therapies, reducing the lifetime costs associated with disease treatment and allowing affected patients to return to work and live independent lives. This is not only invaluable to patients, but saves billions of dollars in supportive care and long-term disability.

Currently, septic shock costs the Canadian healthcare system an estimated \$4 billion annually. There is no treatment for septic shock beyond supportive care and it often results in long-term hospital stays, the need for ongoing treatment or, more commonly, death.

As mentioned previously, OIRM funded Dr. Lauralyn McIntyre's groundbreaking phase I clinical trial in 2017, which showed the safety of her cell-based therapy for this devastating condition. Her project is set to go into a phase II multi-centre trial

in late 2018 to showcase the efficacy of this treatment. OIRM funding is helping to support this multi-centre international trial by funding the cell scale-up technology being done at the Ottawa Hospital Research Institute. This project, while providing a potential cost savings to the healthcare system, will also create jobs in cell manufacturing.



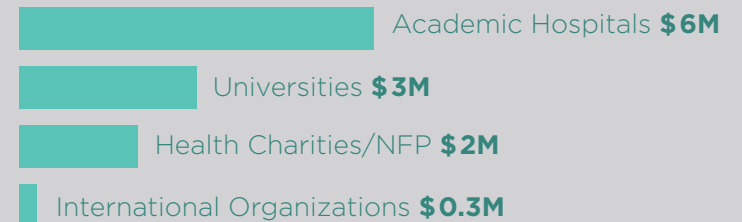
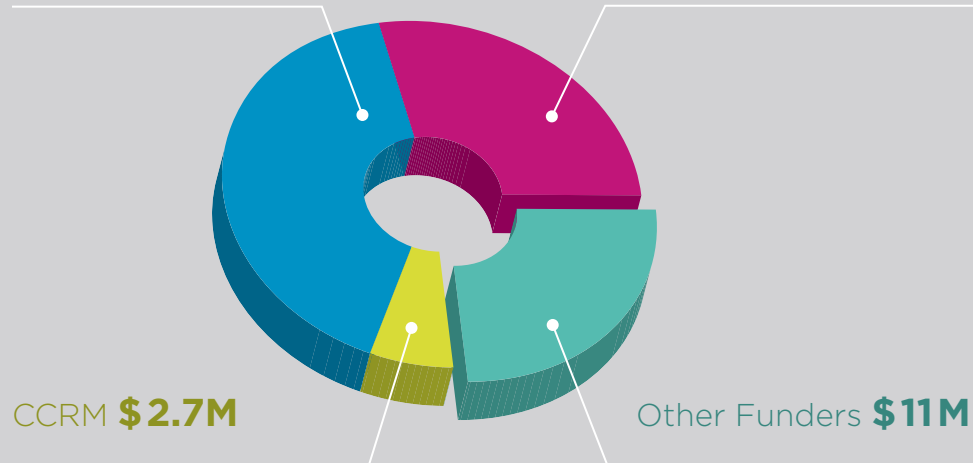
Dr. Freda Miller at the Hospital for Sick Children in Toronto offers another example. Miller and her

team are researching the use of the generic drug metformin for endogenous white matter repair of the brain in youth. Because this treatment takes place early in the lifespan and uses a commonly available drug, it has the potential to have a substantial long-term impact in the health of those with multiple sclerosis, at a greatly reduced cost in comparison to current treatments.

# OIRM leveraged funding success

Federal Government **\$18M**

Private Sector/Industry **\$12M**



In 2017-2018, OIRM was able to leverage its investment of \$6.75M to \$44.7M in leveraged funding (over 6-fold!). \$3M of OIRM's budget went towards supporting CCRM's Commercialization and Manufacturing Platforms and \$3.5M went directly towards OIRM's Disease Team and New Ideas grant programs.

The success of these programs would not be possible without significant investment from OIRM's many strategic partners, including universities, academic hospitals, health charities, industry, venture capital and other non-profit funding agencies.


OIRM investigators secured industry support from 23 companies, including many large global partners such as Fresenius Kabi, Stem Cell Technologies, Milenyi Biotech and Biological Industries.

This speaks to the international recognition of the Ontario's research strengths and the value these industry partners place on engaging with these promising technologies. In addition to industry support, five health charities directly supported these research projects, helping to accelerate bringing these therapies to clinic where they can directly benefit patients.

OIRM also developed partnerships with Medicine by Design (University of Toronto) and Stem Cell Network, two federally funded partners, to co-fund OIRM New Ideas and Disease Team programs respectively. These partnerships secured over \$900,000 in additional support for OIRM funded projects.



# OIRM researchers generate new projects that are priming the pipeline



The OIRM New Ideas program supports projects at the discovery level in the area of stem cell and regenerative medicine research. These projects are typically based at one institution with one or two investigators and have clear and achievable short-term goals leading to clinical or commercial activities.

These New Ideas are the discovery arm for OIRM. Many of our current Disease Teams started out as discovery level projects that continued to evolve towards clinical translation.

Researchers like Dr. Jenny Bruin from Carleton University, Dr. Penney Gilbert

from the University of Toronto and Dr. Michael Rudnicki from the Ottawa Hospital Research Institute are able to take on innovative projects due to these funds.

In 2017-2018 OIRM supported seven New Ideas projects of \$75,000 value each.

**Read about Jenny Bruin's research in this OIRM Research Profile.**

**Read about Penney Gilbert's research in this OIRM Research Profile.**

# OIRM developed industry partnerships



For 2017-2018, OIRM developed an industry partnership program focused on engaging industry stakeholders as active partners in the regenerative medicine community.

For grant funding bodies, industry partnerships are a pivotal part of moving research and technology closer to the bedside. By helping Ontario investigators develop relationships and partnerships with industry, the Institute is supporting faster implementation of therapies and greater opportunities to move towards commercialization.

OIRM connected with more than 30 companies over the last fiscal year to ensure that the research community had the support of networks beyond grant funding bodies. Through these partnerships OIRM has been able to leverage support for both funded and non-funded researchers.

As an example, during the latest CIHR/NSERC Collaborative Health Research

Project competition, OIRM introduced Dr. Erik Suuronen from the Ottawa Hospital Research Institute to our partners at Bio-technie. This meeting helped to secure OIRM specific pricing on supplies for Dr. Suuronen and his team that will provide significant cost savings over the duration of this three-year grant. Additionally, the investigator was able to leverage his affiliation with OIRM to secure an equipment purchase that the group would otherwise have been unable to afford.

OIRM has also developed three formal industry discount programs with Bio-Techne, Roche and Peprotech, which allow OIRM member investigators to purchase supplies and reagents at a greatly discounted price. Partnerships such as these are highly favourable to many granting agencies, as they ensure more of

the grant is spent on direct research and less on supplies. OIRM is pleased to be able to facilitate these connections.

Thirteen industry partners joined the OIRM community at our workshops and annual symposium during the last fiscal year, ensuring OIRM investigators have the most up-to-date information about products and industry trends. OIRM also hosted a workshop focused on the importance of investigators partnering with industry. This highlighted the practical point that when investigators establish these relationships they can better customize their purchases, saving both time and money. The opportunities around the infrastructure support that many companies can provide when moving lab-based technologies into the clinic was also discussed.



## OIRM inspired young minds

OIRM sponsored more than 15 events in 2017/2018, including Stem Cell Talks in Toronto, Hamilton, Guelph, London and Ottawa. This program educates high school students about stem cells and brings in top researchers in the field, supported by a team of dedicated graduate student volunteers, to engage young minds in conversations about topics like stem cell ethics, neural stem cells and more.

In addition, OIRM supported the University of Toronto Chapter of the Student Society for Stem Cell Research (SSSCR). This organization was founded in 2003 and spans over 10 countries and over 100 institutions. Its mission is to inform undergraduates on the promise of stem cells and encourage students to pursue graduate training in the STEM field.

OIRM sponsored their annual conference: Stem Cells: Frontiers, held in Toronto on Jan 27, 2018. This event was attended by 200+ students interested in careers in research, in particular in the field of stem cells and regenerative medicine. The conference featured 16 speakers from a diverse range of professions, including professors, researchers,

physicians, lawyers and ethicists. Six OIRM investigators spoke at the event, as well as Dr. James Till, co-discoverer of stem cells. OIRM also supported SSSCR's tour day, which hosted 150+ high school students at the University of Toronto and provided laboratory tours and experiment demonstrations.

Photo: courtesy of Stem Cell Talks Toronto

# OIRM's voice grew online and in person



In 2017-2018, OIRM continued to have a robust and engaged following on Twitter and the organization launched new Facebook and LinkedIn pages to reach new audiences and better tell the stories of the regenerative medicine research being funded in Ontario. These new channels are growing steadily with a plan to move our Think & Link Rounds series to Facebook Live in 2018.

OIRM launched two new Stem Cells Inked videos in 2017-2018, as well as running a successful Stem Cell Awareness Day campaign on social media.

**Watch our Stem Cells Inked videos now!**

In addition to a strong online presence, OIRM hosted 28 seminars and events in 2017-2018, bringing some of Ontario's best regenerative medicine researchers to Toronto to host Think & Link Rounds, workshops and seminars. OIRM also

partnered with the University of Toronto's Medicine by Design on their successful Global Speaker Series, which brings internationally recognized speakers in regenerative medicine to Toronto.

# OIRM has so much more growing to do!

OIRM by the numbers for 2017-2018

**256** OIRM Members, including stem cell biologists, biomaterial engineers and translational clinicians, reaching a network of **2,000+** individuals

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## The Investment: **\$3.5M**

in support of  
**5** Disease Teams,  
**7** New Ideas Awards and  
**2** Postdoctoral Fellowship Awards

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## The Leverage: **\$44,709,600**

in leveraged funding

## The Business: Company and job creation stemming from Ontario capacity

- **6** company incubations supported and 2 company relocation to Ontario
  - **16** Ontario-based technologies assessed
  - **8** patent applications; 1 provisional patent
  - **4** active licenses
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**71** partnerships through OIRM's research grants, including private sector firms, universities, hospitals and not-for-profits

**5,000+** individuals engaged through public outreach events

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**186** HQP trained through OIRM grants

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Supporting **5** full-time employees at OIRM and **20** full-time employees at CCRM

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OIRM appeared in **189** news articles in print-based or online publications and had **5,364** social media engagements