

OSCI AND CCRM:

ensuring Ontario's regenerative medicine legacy lives on



Michael May

It's hard to believe it's been 50 years since Drs. Ernest McCulloch and James Till of the University of Toronto and the Ontario Cancer Institute, first discovered stem cells. But Ontario can take credit for being a lot more than just the birthplace of stem cell research.

Ontario researchers have played a role in many other regenerative medicine breakthroughs from Dr. John Dick and his discovery of the first cancer stem cell, to Dr. Gordon Keller who was able to generate three types of heart progenitor cells from embryonic stem cells; or Dr. Andras Nagy for his discovery of a new non-viral method of creating stem cells from other cells of the body. When it comes to stem cell research and regenerative medicine, make no mistake, Ontario is a world leader.

Much of Ontario's success in regenerative medicine can be credited to its tightly knit regenerative medicine community. Every new discovery has forged an even tighter bond, encouraging collaboration and most importantly, the creation of a united front in the form of the Ontario Stem Cell Initiative (OSCI).

"OSCI is about forming linkages and building on a collaborative environment in which we can make new research happen, bring new resources to the table to enable the translation of new treatments for disease," explains OSCI director Janet Rossant.

According to Rossant OSCI has three main goals: to promote better science by collaboration, promote international collaboration and to develop an economic base for regenerative medicine in Ontario. "There is no question that stem cell

research and regenerative medicine are one of the frontiers of science. There's huge potential for us to harness stem cells, whether they're embryonic stem cells, induced pluripotent stem cells or adult stem cells. The ability to harness them to study disease, to understand disease better and to develop new drugs to treat disease will change the way we offer treatment to patients in the future. OSCI allows us to focus on this exciting new frontier in a more focused way. It allows the group better access to provincial funds and ensures that we're working together, rather than against one another," said Rossant.

The first step to forming OSCI was the establishment of The Ontario Human Induced pluripotent Stem (iPS) Cell Facility. Backed by seed funding from the Ontario Ministry of Research and Innovation, the facility is positioning Ontario at the cutting edge of the new technologies and applications in induced pluripotent stem cells. Momentum continued to build and today OSCI is a virtual network of over 60 stem cell scientists across multiple institutions in Ontario including the McEwen Centre for Regenerative Medicine at the University Health Network, The Centre for Stem Cells and Tissue Engineering at Mount Sinai Hospital, The Hospital for Sick Children Research Institute in Toronto, The Sprott Centre for Stem Cell Research in Ottawa, the Advanced Regenerative Tissue Engineering Centre, the McMaster Stem Cell and Cancer Research Institute in Hamilton, the University of Toronto and the University of Western Ontario.

With the ability to collaborate and talk to each other, Rossant says OSCI has been able to avoid redundancy in research, but more importantly, the group has been able to build mega programs out of its research.

"OSCI has allowed us to take things to a whole new level. Two examples of great projects facilitated through Ontario government financial support (Ontario Research Fund Global Leadership Program) and OSCI research collaboration include one led by Dr. Andras Nagy generating pluripotent stem cells and the other led by Dr. Mick Bhatia screening stem cells for molecules that can promote formation of

differentiated cells. Both of these projects have produced significant findings that likely wouldn't have been possible without OSCI," she said.

In addition to facilitating innovative collaborative projects, new research funding has allowed the province to recruit researchers, international students, and post docs from abroad to its centres.

"There are young scientists joining research institutes in London, Hamilton, Ottawa and in Toronto," says Rossant.

With the formation of OSCI, the whole has become greater than the sum of its parts.

CCRM: A NEW CHAPTER IN ONTARIO'S STEM CELL STORY

As mentioned, developing an economic base for regenerative medicine is one of the main goals of OSCI. So, if OSCI is about bringing Ontario's regenerative medicine community together, then the Centre for Commercialization of Regenerative Medicine (CCRM) is about capitalizing on this collaborative success in the commercialization arena.

It's no secret that most of the intellectual property that comes out of academic institutions is often very early stage. CCRM has been created to prevent the premature creation of companies and licensing of technologies, and to take that IP and advance it through the product development stage in a capital efficient way.

"Particularly, CCRM will work towards filling gaps in the pipeline from discovery to application and to provide the tools and targets to fill those gaps. CCRM recognizes the importance of large scale clinical trials with stem cells, but is focusing on enabling technologies that will allow stem cell research to really impact on disease therapies," explains Rossant.

In turn, the companies that traditionally steered clear are now finally realizing regenerative medicine is an extremely important area to invest.

"Biopharma stands to benefit from the efforts of CCRM and advancements in the regenerative medicine. For example, our ability to make stem cells and specialized cell types from patients with many different forms of disease and grow those in petri dishes and grow those in large numbers allows us new tools to screen



for new treatments, new diagnostics, new prognostics and new drugs to treat different diseases," said Rossant.

CCRM chief science officer Peter Zandstra explains that CCRM will concentrate its efforts on foundational bottlenecks. One example he gives is developing better ways to grow cells in large scale and more efficiently.

"There are different resources that the community needs to accelerate translation, whether this is resources for characterizing cells, resources for producing those cells, or sources of disease specific models for high throughput drug development, these are just some of things we'll be targeting," he said.

As such, CCRM will focus its effort on three platforms to start. Firstly around reprogramming and creating a library of patient and disease specific cell lines, secondly around cell manufacturing with an aim to produce clinical and commercial quantities of differentiated cells and thirdly biomaterials and tissue mimetics.

According to CCRM CEO Michael May, what sets CCRM apart from other commercialization groups is that it provides resources (Ontario's reputed stem cell community and generous funding), facilities (both academic and institutional), expertise (business and product development professionals) and a consortium of private companies or members ready to be receptors for new technologies produced through CCRM efforts.

"There are few jurisdictions in the world that combine our expertise in stem cell biology, bioengineering and biomaterials. We think this is a unique translational model that leverages our academic facilities and resources. But those of us who have tried to move technology forward understand that technology push which we're so comfortable with in academia, needs to be balanced by market pull. So we've built an industry consortium of 15 to 20 companies that are ready receptors for the Intellectual Property (IP) we're moving forward. They can inform our decisions on where to focus," he said.

He adds that these companies are large and small, domestic and international, and they represent all aspects of the regenerative medicine industry.

In terms of expertise and business leadership, CCRM's board of advisors is heavily focused and dominated by seasoned industry veterans in regenerative medicine. They understand how IP might be packaged and positioned to

start new companies or to be licensed to existing companies.

The end goal of CCRM is to create a critical mass of resources for company creation and product development, or an environment where the regenerative medicine industry can germinate, while at the same time supporting the entrepreneurial activities of researchers.

"Through CCRM, we are building a network of receptors for their IP or for the companies that are created from their IP

that will situate themselves in Canada.

In some cases the IP will just go to one of our consortia partners because they've helped pay for it, they're supportive and they need that technology. In other cases we'll build companies around that IP with the idea that these companies will be viable in Canada. Ultimately, through CCRM's efforts, we will bring entrepreneurs and young scientists to Ontario and over the long term, money, investment and capital back to the regenerative medicine community," states May.



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