JDRF Canadian Clinical Trial Network

Life-changing innovation to defeat type 1 diabetes

JDRF IMPROVING LIVES. CURING TYPE 1 DIABETES.

Executive Summary

- Established in 2009 with $20 million in funding from FedDev Ontario and $13.9 million from JDRF, the JDRF Canadian Clinical Trial Network (JDRF CCTN) serves as an incubator and accelerator for clinical trials aimed at curing, treating and preventing type 1 diabetes (T1D).
- JDRF CCTN fosters innovation and collaboration by bringing together researchers from dozens of provincially-funded hospitals and research institutions to run multi-site clinical trials in Canada, helping to spread and scale the most proven and promising approaches by bridging the gap between fundamental research and commercialization.
- JDRF CCTN over-delivered on its commitments, funding 12 leading-edge clinical trials and creating 266 good quality jobs in the research sector. In addition, the network leveraged more than $5 million in additional funding and in-kind donations, expanding the scope of the research, with new trials in Alberta and BC and creating seven new fellowships for promising young researchers.
- Canadian researchers now participate in up to 19% of the global clinical trials JDRF is funding worldwide even though Canada has approximately 2% of the global population.

The Proposal

{new clinical trials}

{200+ jobs}

JDRF is requesting a $25 million commitment from the Government of Canada and will commit $15 million in additional funding from Canadian donors. This funding will allow us to scale up JDRF CCTN nationally and leverage additional funding from industry partners, bringing its benefits to all Canadians. Having successfully established a research pipeline, JDRF CCTN has the infrastructure in place to identify projects and disburse funds within the next 6-9 months.

New funding will make possible up to 10 new clinical trials across Canada, accelerating the pace of critical research, creating hundreds of new highly-skilled, knowledge-based jobs and ensuring that Canada continues to be a centre of excellence for diabetes research.

Government funding now will help *JDRF CCTN unlock exciting new breakthroughs* in how this extremely challenging disease is treated; breakthroughs that promise *innovation in service delivery*, *improved outcomes and quality of care* for the more than 300,000 Canadians with T1D and their families (and in some cases for the millions of Canadians with diabetes – type 1 and type 2) as well as *drastically reduced costs* for Canada's health care system and out-of-pocket costs for families.

About T1D

Type 1 diabetes (T1D) is a devastating, potentially fatal, autoimmune disease in which a person's pancreas stops producing insulin, a hormone that transforms food into energy. T1D strikes children and adults suddenly, causing dependence on insulin for a lifetime, and carries a constant threat of devastating complications, which may include: kidney failure, blindness, nerve damage, amputation, heart attack, stroke, pregnancy complications and the risk of dangerous high or low blood sugar levels, both of which can be life-threatening. Unlike type 2 diabetes, diet and lifestyle factors are not a trigger for the sudden onset of T1D in children or adults.

Canada has a rich legacy of innovation in T1D research. From the world-changing discovery of insulin by Sir Frederick Banting and Dr. Charles Best to the Edmonton Protocol to new areas of research such as encapsulation and the artificial pancreas project. Canadian researchers today stand on the cusp of new and exciting discoveries which will improve the lives and health of Canadians living with T1D. Despite these scientific advancements, Canadians face mounting personal and public costs brought on by diabetes, which are expected to increase to $16.9B by 2020 – the equivalent of the entire 2014/2015 health care budget of the province of British Columbia.

{Dr. Frederick Banting}

Background

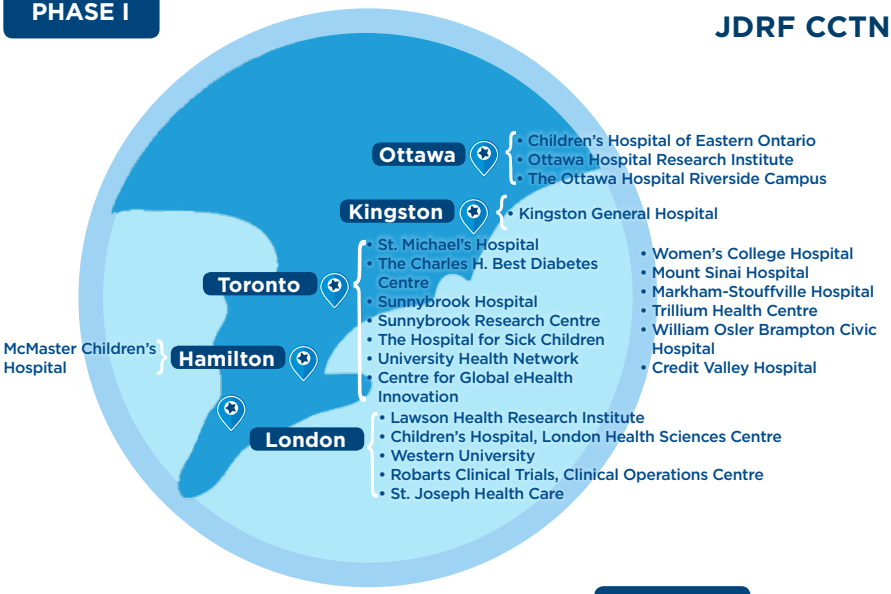
In 2008, the House of Commons Standing Committee on Finance recommended that the government of Canada support the development of a Canadian Clinical Trial Network in partnership with JDRF to accelerate the pace of diabetes research in Southern Ontario. This partnership marks a significant first step in ensuring that Canada continues to be a premier hub and home for internationally-recognized, cutting-edge diabetes research.

The JDRF CCTN was established in 2009 with $20 million in funding from FedDev Ontario and $13.9 million from JDRF as an incubator for clinical trials aimed at curing, treating and preventing Type 1 Diabetes. By bringing together researchers from dozens of provincially-funded hospitals and research institutions to run multi-site clinical trials, JDRF CCTN fosters innovation and collaboration, helping to bridge the gap between fundamental research and commercialization by carefully vetting projects to help spread and scale the most proven and promising approaches.

JDRF CCTN's success in Ontario helped JDRF leverage additional funding, including an additional $1 million in technology and staff expertise from Medtronic, $3 million from the WB Family Foundation which allowed the network to expand into western Canada, launching Phase II of JDRF CCTN with new trials in British Columbia and Alberta, $700,000 from JDRF International and $400,000 from Lilly Canada to create seven new fellowships, advancing the training of young researchers.

When the original partnership agreement with FedDev Ontario was signed, the expectation was that three clinical trials would be undertaken and 152 jobs created. JDRF CCTN exceeded all expectations, creating 266 jobs in the knowledge-based research sector and helping Ontario and Canada to attract and retain the best and brightest research minds. To date, 12 peer-reviewed, leading-edge clinical trials have been funded. In addition, a digital technology project is developing global standards for insulin pumps and continuous glucose monitors; standards we believe will lead to higher adoption rates for life-saving technologies, improving diabetes management and ultimately reducing complications and health care costs.

PHASE I

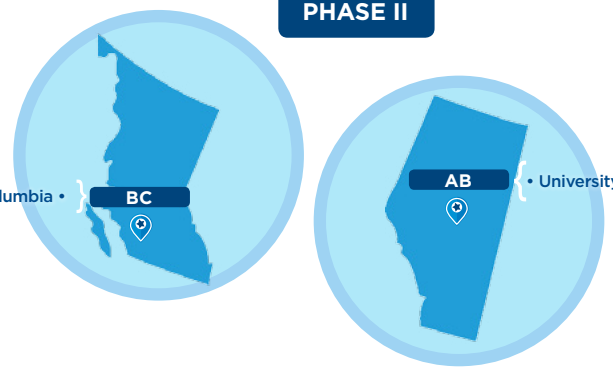


JDRF CCTN

- Creates an infrastructure for diabetes research.
- Increases Canada's competitiveness in attracting global research dollars.
- Unlocks exciting new breakthroughs, leading-edge treatments and technologies.

PHASE III

PHASE II



Delivering Life Changing Results for Canadians

With this $33.9 million in funding, CCTN has demonstrated remarkable benefits and capabilities in innovative research and has contributed to improving the health of Canadians living with T1D. This includes:

- Twelve peer-reviewed clinical trials in Ontario plus one technology project, significantly more than the three trials originally envisaged.
- Creating and sustaining 266 highly-skilled, knowledge-based jobs across leading hospitals, academic, medical and health science centres and industry, more than the predicted 150 jobs.
- Providing nearly 1,000 Canadians with T1D with early access to life enhancing technologies or solutions through these clinical trials.
- Advancement of leading-edge research which would pave the way for
    - ◊ development of encapsulation technologies which could provide a cure for diabetes, introducing insulin-producing beta cells into the body while protecting them from immune response, eliminating the need for daily insulin injections;
    - ◊ improved maternal health outcomes by reducing congenital defects and birth injury;
    - ◊ better diabetes management for adolescents, reducing incidents of hyper- and hypo-glycemia;
    - ◊ preventing the devastating complications, including vision loss, nerve damage and kidney disease, associated with diabetes (Type 1 and Type 2).

Since its launch, CCTN has grown into an international hub for T1D translational research, innovation, and global leadership of new therapeutics and enabling technologies As a result of the Government of Canada's investment, Canada is now home to a significantly larger portion of JDRF's funding for global trials. Canadian researchers now participate in up to 19% of the global clinical trials JDRF is funding worldwide even though Canada has approximately 2% of the global population.

Case Study

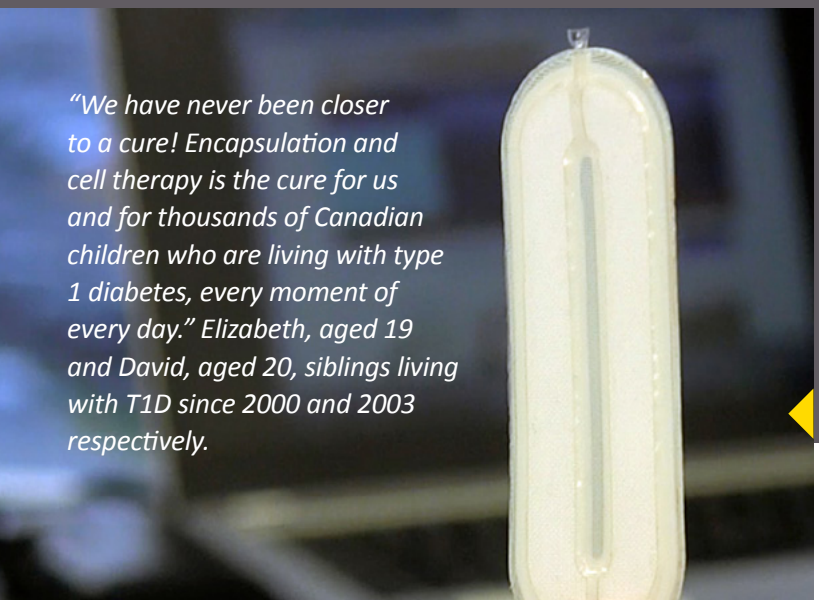
ViaCyte Encapsulation Trial

The highly anticipated STEP ONE (Safety, Tolerability, and Efficacy of VC-01 Combination Product in Type One Diabetes) encapsulation trial came to fruition at its second site in 2015 at the University of Alberta (U of A) in Edmonton. The purpose of this research is to assess the safety, tolerability, and effectiveness of a new encapsulated delivery system that will protect insulin-producing pancreatic beta cells.

The encapsulation device holds pancreatic progenitor cells that are produced by a proprietary method from a stem cell line. Implanted under the skin of the patient in an outpatient surgical procedure, the enclosed cells develop into mature cells that secrete insulin and other factors, thereby regulating blood sugar levels and reducing or completely eliminating the need for insulin injections.

U of A's lead investigator for the trial is Dr. James Shapiro, globally-renowned for having developed the Edmonton Protocol for the transplant of pancreatic islet cells as a treatment for T1D.

> *"This is potentially much closer to a cure for diabetes than what we've been doing up to this point.*
>
> Dr. Shapiro, Global News, December 15, 2015



"We have never been closer to a cure! Encapsulation and cell therapy is the cure for us and for thousands of Canadian children who are living with type 1 diabetes, every moment of every day." Elizabeth, aged 19 and David, aged 20, siblings living with T1D since 2000 and 2003 respectively.

{Left, VC-01 encapsulation device}

Case Study

CONCEPTT Trial

Women with type 1 diabetes continue to have adverse pregnancy outcomes, including high rates of major congenital malformations, stillbirth, and neonatal death. Pregnancy is one of the biggest accelerators of diabetic complications.

This CCTN study is a randomized, open-label controlled trial funded by JDRF to evaluate the benefit of using a real-time continuous glucose monitor (RT-CGM) sensor for pregnant women and to improve baby outcomes. The sample size is 324 women (110 pre-pregnant and 214 pregnant), to be recruited from eight centres in Canada and 10 internationally.

This Canadian based trial is supporting an international group of more than 20 leading investigators in the U.S., Europe, and Israel.

> *"Through all the years of discussion around pregnancy,I knew it was a big deal, On average, you need to start working with your diabetes team up to a year before you're planning to conceive... It's a lot of pressure when you know that not only your own quality of life depends on that control, but also the health of your child."*
>
> Vanessa Oliver, diagnosed at age six with T1D and a participant in the CONCEPTT trial

Institutional Partner	Principal/Site Investigator
Mount Sinai Hospital, Toronto, ON	Dr. Denice Feig
Sunnybrook Health Sciences Centre, Toronto, ON	Dr. Julia Lowe
St. Joseph's Health Care, London, ON	Dr. Ruth McManus
Kingston General Hospital, Kingston ON	Dr. Robyn Houlden
St. Michael's Hospital, Toronto ON	Dr. Maria Wolfs
McMaster University Medical Center, Diabetes Care & Research, Hamilton, ON	Dr. Natalia Yakubovich and Dr. John Booth
The Ottawa Hospital, Riverside Campus, Ottawa, ON	Dr. Erin Keely
Foothills Medical Centre, Diabetes in Pregnancy Clinic, Calgary , AB	Dr. Lois Donovan

> *"With trials such as CONCEPTT, we are positioning Canada as an international hub for diabetes science and innovation, researching new technologies and therapeutics that will enable the diabetes community to lead better, longer and healthier lives."*
>
> Dave Prowten, President & CEO, JDRF Canada

About JDRF Canada

Founded by parents of children with type 1 diabetes (T1D), JDRF is the leading global charity focused on research that would cure, prevent & treat the disease. For more info visit jdrf.ca

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