Spring 2018

Donor Report

Huntington Society of Canada Société Huntington du Canada

"Nothing is impossible, the word itself says 'I'm possible'!"

- Audrey Hepburn

This report is for our caring, passionate donors who pull together and change the reality for families living with Huntington disease. You inspire us each and every day by providing practical help for families grappling with HD and investing in research to slow and prevent this disease. This report is for you and highlights some of the recent accomplishments you made possible. Thank you for your leadership and support. You are providing hope and a positive view of the future for families impacted by Huntington disease.

Impact of HSC Donors in 2017

GENETIC NON-DISCRIMINATION ACT BECAME LAW

NEW RESOURCE CENTRE OPENED IN NEW BRUNSWICK

30% MORE YOUTH CONNECTED THROUGH OUR YOUNG PEOPLE AFFECTED BY HUNTINGTON DISEASE DAY

1.3 MILLION DOLLARS COMMITTED TO **5** HD RESEARCH PROJECTS

3 GLOBAL HD ORGANIZATIONS INCLUDING HSC, FORMED HDCOPE*

*community members informing HD clinial trials

8 HD CLINICAL TRIALS ACTIVE IN CANADA WITH KEY TRIALS ADDRESSING THE ROOT CAUSE OF HUNTINGTON DISEASE

155% INCREASE IN ENGAGEMENT & EDUCATION THROUGH SYMPOSIUM

FAMILY SERVICES WORKERS CONTINUE TO SUPPORT OVER **15,000** PEOPLE AFFECTED BY HD

99 GLOBAL LOCATIONS PARTICIPATED LIGHT IT UP 4 HD

RESEARCH

HSC DONOR-FUNDED RESEARCH REDUCES HD SYMPTOMS IN MICE

Because of the support from donors like you, HSC was able to fund research that was conducted by Dr. Stephen Ferguson, from the University of Ottawa Brain & Mind Research Institute. His research has shown to reduce HD symptoms in mice. Dr. Ferguson's research uses Roche Pharmaceuticals CTEP inhibitor which also reduces Huntington pathology in the brains of treated mice.

"This is an exciting time in Huntington disease research," explains Bev Heim-Myers, CEO. "For the first time ever, human clinical trials are taking place that address the root cause of Huntington disease. Dr. Ferguson's research is complementary and adds value to current research that is in clinical trials, as it uses a separate pathway to deliver the treatment; as such it could be used in conjunction with antisense oligonucleotides (ASOs). Innovative research initiatives, such as the work being done by Dr. Ferguson and his team, have the potential to transform HD research. The answers we find for Huntington disease will likely lead to a better understanding of treatments for other neurological diseases such as Parkinson's, Alzheimer's and ALS."

Dr. Ferguson's research was funded through HSC's Navigator Research Program which is made possible by support from our generous donors like you. This program supports basic scientific research in Canada that is of direct and immediate relevance to Huntington disease research globally. The aim of the research program is to provide a platform for the recruitment of outstanding investigators to HD research, to facilitate research collaboration nationally and internationally and to support research that is relevant to other neurodegenerative disorders.

For more information on Dr. Ferguson's research, visit www.HDResearchNews.ca.

HUNTINGTIN LOWERING TRIAL UPDATES

Thanks to HSC donors, Canadians have more opportunities than ever to take part in clinical trials for potential HD treatments.

Donor support allowed for the creation of the Clinical Trials Consortium which first convened in 2015. Since then this group has worked together to connect clinicians, share best practices and maximize the opportunities for people affected by HD to participate in clinical trials.

Support from HSC donors helped to lay the foundation for these trials to take place, and this will only prove more beneficial in the future as more trials become available.

IONIS-HTTRx

In December, Ionis Pharmaceuticals announced that the HTTRx drug has successfully lowered the harmful huntingtin protein in cerebral spinal fluid. The drug is safe and well tolerated. This announcement is promising and gives us substantive hope that a treatment is near. The Canadian HD community has been leading the charge. Canadians are a part of this clinical trial, helping us all to get to this important point in our history.

What happened?

- On December 11, 2017, Ionis Pharmaceuticals announced the results of a global Phase 1 clinical trial that tested an HD drug called IONIS-HTTrx.
- This therapy is designed to target the root cause of HD.
- This Phase 1/2a trial took place in Canada and Europe. It was a small trial, with about 40 participants.

What were the results?

- The therapy was well tolerated, and there were no safety issues.
- Reductions in the toxic mutant huntingtin protein were observed in the study participants.
- This is promising news. The therapy is designed to lower the mutant huntingtin. The press release indicates that huntingtin lowering has been achieved.
- Important Notes:
    - This study was short. Each patient only received four months of injections. This is not enough time to look for changes in the rate of HD progression.
    - At this point, we do not know if this drug had an effect on participants' HD symptoms.

What is next?

- Roche Pharmaceuticals will take on the licensing of IONIS-HTTRx, assuming responsibility for all future developments. Roche has expertise in developing medicines to treat neurodegenerative brain diseases and has experience bringing these medicines to patients.
- The next step for this program is to continue the trial to later stages with more participants. Researchers will investigate if reductions in the mutant huntingtin protein benefit people with Huntington disease by favourably impacting HD symptoms.
- Our expectation is that the next phase of this trial will start in late 2018 or early 2019.
- This will be a global trial. The trial sites have not yet been confirmed, but our hope is that more sites will be offered in Canada.

WAVE PRECISION-HD

Dr. Mark Guttman described in his Symposium presentation in October that two more potential HD treatments have reached the point of clinical trials, and his Toronto clinic is the first in the world to be testing them.

Wave Life Sciences has developed two closely related drugs – WVE-120101 and WVE-120102 – that are designed to stop cells from making the mutant huntingtin protein that is thought to be toxic to the brain. Like the IONIS-HTTRx trial, Wave's PRECISION-HD trials are testing drugs that "shoot the messenger" – targeting the messenger RNA (mRNA) that translates the HD gene and leads to the production of mutant huntingtin protein. By reducing the messenger, these drugs aim to stop the disease in its tracks or prevent it from developing in the first place. However, unlike the IONIS drug, the Wave drugs target only the RNA that is associated with the abnormal CAG repeat, leaving the normal part untouched.

The first doses have already been given to courageous trail blazers in Toronto! There's a long list of criteria they need to meet to be eligible, however.

As soon as we have information on the next phase of the trial, we will post it on our website at www.HDLoweringTrials.ca.

To learn more about HSC research investment programs visit www.huntingtonsociety.ca/research.

FAMILY SERVICES

Over 15,000 individuals across Canada are supported by our Family Services team. This team delivers services from coast-to-coast, ensuring that individuals, families and organizations are educated about HD. Services include individual, couple and family support, advocacy for services where gaps are identified and support groups across the country. Thanks to donors like you, the Family Services team also provides youth specific services through a Youth Mentorship Program. This program connects youth growing up in a home impacted by HD, with a HSC-trained young adult that can provide insight on how to navigate life choices in light of HD. This is made possible by the ongoing and continued support of our donors. Thank you.

CONNECTING MORE FAMILIES ACROSS CANADA

Because of the generous support of donors like you, more individuals and families affected by HD are receiving the support they need in communities across Canada. Since September, over 60 support group sessions were held to offer families a safe place to discuss their HD struggles. Our Family Services team members were also able to accompany clients to over 400 clinic appointments and provide 33% more HD in-services sessions to care facilities to create better care for our families.

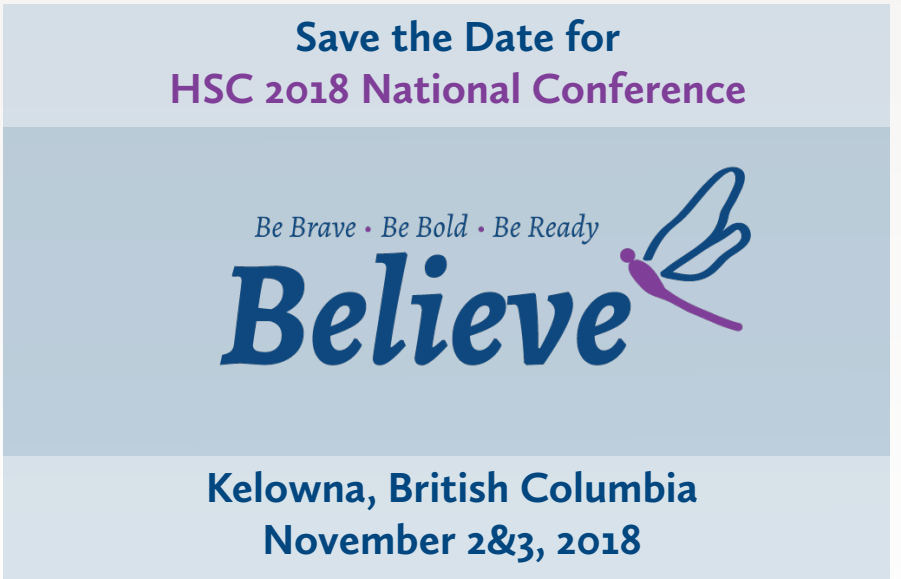
HD COALITION FOR PATIENT ENGAGEMENT (HD-COPE)

HD-COPE is a new international collaboration between the Huntington Society of Canada, the Huntington Disease Society of America (HDSA) and the European Huntington Association (EHA). HD-COPE was formed to replace the current ad hoc approach to incorporating the patient-voice in global therapeutic development efforts for HD. The group's role is to communicate the HD community's experiences and needs to regulators, industry and researchers. In February 2018, the first HD-COPE meeting was held in London, England with four Canadians, six Americans and 10 Europeans who are personally affected by HD. The meeting included clinical trial training, a visit to a research lab and a meeting with a pharmaceutical company to give family members impacted by HD the opportunity to provide first-hand knowledge of the challenges of living with HD. The support of donors like you has helped to ensure that this is the first of many interactions for the group and that it will grow over time to include more family members and HD organizations from around the world.

For a complete list of Family Services team members, and to learn more about the support our Family Services team provides, please visit www.huntingtonsociety.ca/family-services-program.

CONNECTING OUR COMMUNITY

HUNTINGTON SOCIETY OF CANADA 2018 NATIONAL CONFERENCE



Through donor support, we are able to meet and connect face-to-face with our community of individuals living with or at-risk for Huntington disease, including youth, caregivers, researchers and care professionals on a biennial basis at our HSC National Conference. This year, Conference will be held in Kelowna, British Columbia on November 2 and 3.

2017 YOUNG PEOPLE AFFECTED BY HUNTINGTON DISEASE DAY (YPAHD DAY)

As part of our Youth Program, and through the support of donors like you, HSC supports our virtual youth Chapter in holding Young People Affected by Huntington disease (YPAHD) Day annually. YPAHD Day was hosted on November 18, 2017 in Moncton, Toronto and Kelowna.

More than 70 youth attended this educational event which is a 30% increase over the previous regional YPAHD Day. Over 75% of the youth were fully funded thanks to donor support, and 46% were first time attendees.

One YPAHD Day attendee, Jen, said, "This was a place that everyone felt like they belonged – a place where you could feel normal. In some families, like my own, the HD conversation is not an easy one to have. It's also something that some families tend to keep as a secret. YPAHD Day 'normalized' HD. People could mingle, share and ask questions with others. Connections and friends were made that day."

To learn more about YPAHD visit www.ypahd.ca.

HSC 2017 NATIONAL SYMPOSIUM

Every other year, thanks to the generosity of our donors, people affected by HD, medical professionals and support professionals across Canada come together for a one-day HD educational event. Together, we learn, inspire one another, and offer support. Working with volunteers from across Canada, HSC hosts a variety of locations from coast-to-coast.

On October 14, 2017, 500 people attended one of 18 Symposium locations across Canada, or chose to view from home with our virtual streaming option. Symposium saw a 155% increase in participation compared to 2015! Viewers joined us from all across Canada and as far as Dubai, New York and Florida.

Through a follow-up survey, we learned that participants "enjoyed being together with other HD families, felt a sense of community, formed bonds with other individuals and felt as if they were not alone."

To view the recorded presentations and download the summaries visit www.huntingtonsociety.ca/symposium.

Thank You

On behalf of families living with HD, thank you for your continued generosity and partnership. Your donations make all the difference as we support families and youth from coast-to-coast, reach out to families who are not yet receiving much-needed support, invest in world-class research and play a leadership role in the international Huntington community.

With your help, we continue to improve the quality of life for people with HD, cultivate strength and resilience in the Huntington community and provide substantive reasons for hope.