



# 2019 FALL DONOR REPORT

How your contributions are helping  
families living with Huntington disease.

# RESEARCH

Twenty-five years after the discovery of the Huntington disease (HD) gene, investigators are now testing drugs specifically designed to address the root cause of Huntington disease. Today, your donations help us continue to invest in the most promising laboratory and clinical research, developing and testing more potential ways to reverse, slow or prevent the progression of HD.

## NAVIGATOR AWARDS

Through our NAVIGATOR Research Program, we fund basic scientific research which creates insights into HD and attracts outstanding investigators to the field.

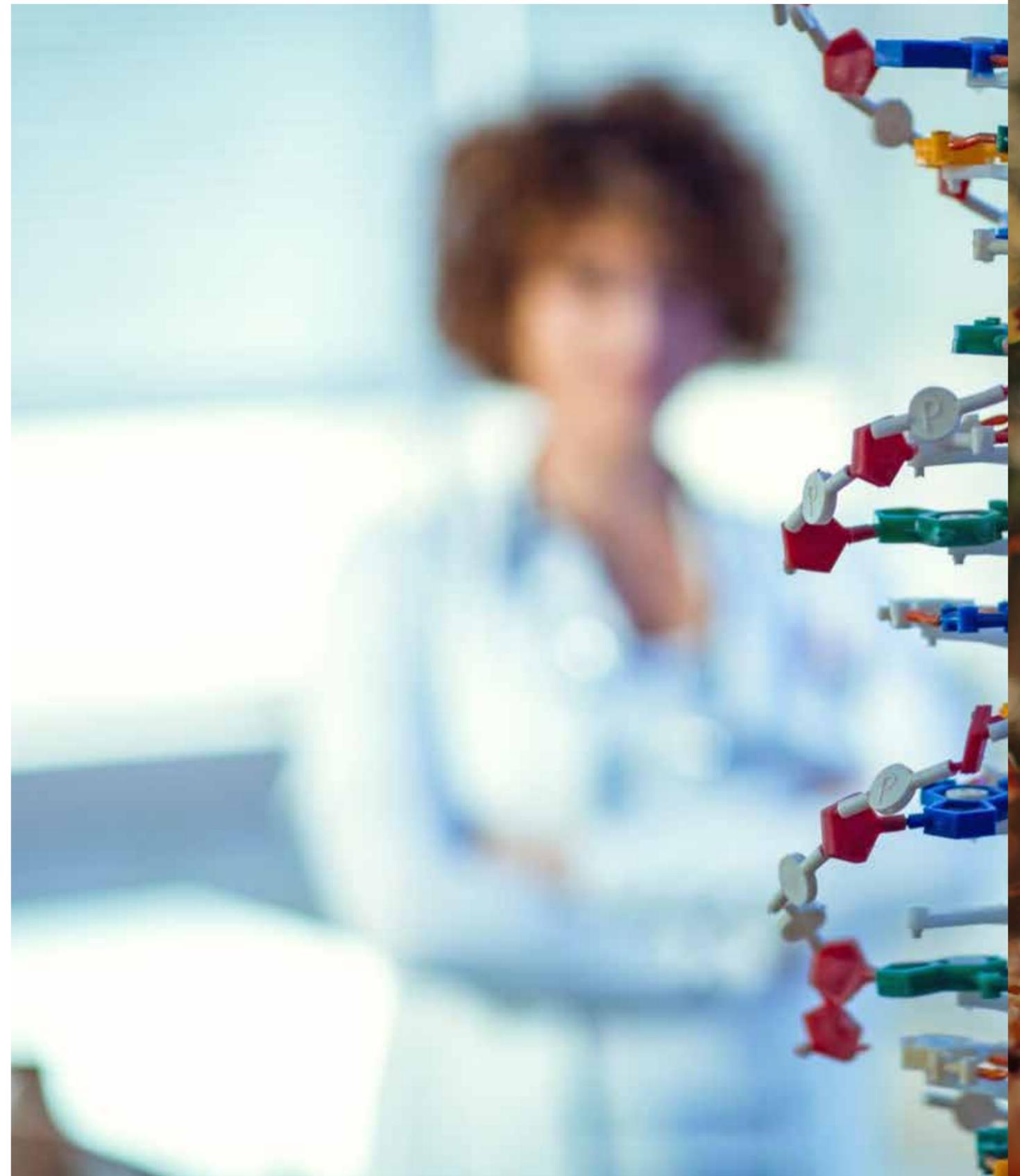
CRISPR gene-editing technology made headlines around the world when it was developed seven years ago. In June, HSC provided the first year of funding for a project which applies that technology to HD. Université Laval's Dr. Francesca Cicchetti and her colleagues in Wales, UK and Boston, MA are using a harmless virus to transport a tiny pair of molecular "scissors" and a guide into brain cells of mice with HD. There, with the help of the guide, the scissors snip away the extra CAG repeats found in the mutant version of the Huntington's gene.

By turning the mutant gene into the healthy version, they aim to stop cells from creating the disease-causing huntingtin protein.

Initial tests in HD mice show that CRISPR can improve movement and cognitive symptoms. Now, with this NAVIGATOR grant, Dr. Cicchetti and her colleagues will conduct a new set of experiments to see whether it reverses HD damage at a cellular level.

They will also test the approach in pre-symptomatic HD mice to see if the promising treatment can prevent the onset of HD. "I think that attacking the DNA itself with these new approaches is very, very exciting," says Dr. Cicchetti. "We're extremely grateful to have the opportunity to test this."

HSC also provided a second year of NAVIGATOR funding for the University of Toronto's Dr. Cheryl Arrowsmith, who is deciphering the three-dimensional structure of mutant huntingtin protein. The results promise to yield important insights into how mutant huntingtin behaves, paving the way for new disease-fighting drugs.



# CLINICAL FELLOWSHIPS:

## Training the Next Generation of HD Specialists

In 2019, HSC launched a pilot program to attract bright young neurologists to the Huntington's field. With the support of the Manning and Beckham families and our generous base of donors, we provided two Clinical Fellowships that offer exposure to clinical research and hands-on training in the diagnosis and management of HD.

### Dr. Ragini Srinivasan

As a newly minted neurologist, Dr. Ragini Srinivasan is keen to hone her clinical skills and learn more about HD. "I hope to get more experience in managing the condition and helping people through their journey with HD," she says.

Working under the supervision of Dr. Mark Guttman, the McMaster University graduate sees patients at the Toronto Centre for Movement Disorders and in North Bay, Sturgeon Falls and Sudbury, ON, where Dr. Guttman makes regular trips to serve families in the northern part of the province.

On the research front, Dr. Srinivasan is conducting physical and cognitive assessments for participants in the Enroll-HD study. Meanwhile, she is also assisting with lumbar punctures in the Roche and Wave huntingtin-lowering trials, testing drugs that have the potential to alter the course of HD. "It's incredible what we're able to do here," she says.

### Dr. Fabricio Pio

Dr. Fabricio Pio's journey began in Brazil, where he received his MD in 1996. He practised as a neurologist there for many years, interspersed with research stints and clinical fellowships in Ontario and Alberta.

In 2018, he joined Dr. Blair Leavitt's team as a research associate at the University of British Columbia's Centre for Huntington Disease, thanks to support from the Manning and Beckman families. During that time, Dr. Pio assisted with huntingtin-lowering trials, while working toward certification with the Huntington Study Group, Roche and the University of British Columbia.

Now fully certified, Dr. Pio is continuing to apply his clinical and research skills to help HD families through HSC's Clinical Fellowship program. Already, his support has enabled the Centre for Huntington Research to accelerate clinical trials, initiate new ones and make life easier for trial participants.

**"This fellowship opportunity has enabled us to train an excellent new HD clinician and to bring them into the fight against HD. It cannot be emphasized enough how important it is for us to train the next generation of HD researchers"** ~ Dr. Blair Leavitt

Thank you for continuing to believe in the power of research. To learn more about how you are moving us closer to a world free from HD, visit [hdresearchnews.ca](http://hdresearchnews.ca).

# FAMILY SERVICES

Each year, your donations help us serve more than 15,000 people affected by HD across Canada — through support groups, one-on-one counselling, education, advocacy and more. Our Family Services Team ensures that individuals, families and professionals have the tools and support they need to face HD at every stage of the disease.

## By the Numbers

15

Resource  
Centre Directors

6

Family Service  
Workers

90

Support Group  
Sessions

Combined 238 Years of Experience

63

Information  
Sessions

11

Drop-In  
Opportunities

85

In-Services for 1250  
Healthcare Staff

# ONLINE SUPPORT

Thanks to your donations, HSC was able to expand our services this year, creating two new virtual ways for families to get the support they need. In June, we launched our Closed Facebook Support Group. Moderated by two of our Resource Centre Directors, this online platform is offering a safe space to share stories, ask questions and access resources. The group already has hundreds of members, with more people joining every day.

Meanwhile, this fall HSC is piloting a Virtual Support Group for people at risk of HD. Like our traditional support groups, each meeting is structured around a particular discussion topic. However, because it takes place via online video conferencing, it is accessible to anyone across the country with a high-speed Internet connection.

Thanks to these new virtual opportunities, families and individuals can now connect with other people in the HD community and get support — wherever they may live and whenever they need it.

To find more information about our Family Services Team or to access a complete list of regional team members, please visit [huntingtonsociety.ca/family-services-program](http://huntingtonsociety.ca/family-services-program).



## CONNECTING OUR COMMUNITY:

### Community Education Forums

Every other year, our Community Education Forums give participants across the country the opportunity to learn about the latest research, connect with others and get their questions answered by HD experts. In the past, HSC has streamed these events live from a central location. This year, however, the keynote speakers were pre-recorded, giving groups the freedom to choose when and where to host their own events.

In the videos, world-renowned HD researchers Drs. Michael Hayden and Ed Wild discuss the different components of a clinical trial, how current studies are going and what's next in this exciting era of Huntington's research. Meanwhile, forum organizers typically complement those videos with live speakers — from local neurologists to HSC Conference favourites like Yvonne Heath and Jimmy Pollard.

The new format has been well received, with participants saying they're getting a much better understanding of the research. Those participants include many people who are connecting with HSC for the first time.

As of September 1, seven sites across the country had already hosted Community Education Forums, and 16 more are scheduled this Fall. And for those who can't attend in person, we are offering a virtual forum on November 2. A huge thank you goes to our sponsors — **Roche, uniQure, Vaccinex and Wave Life Sciences** — and donors like **you**, who have made these events possible.

### Panel Discussion: The Changing Landscape of Huntington Disease

On March 23, 2019 more than 100 people showed up at Toronto's North York General Hospital to learn more about clinical trials.

Our event kicked off with a presentation by genetic counsellor Clare Gibbons regarding the genetics of Huntington's and what genetic testing involves. Next, neurologist Dr. Mark Guttman gave an overview of the HD clinical trials currently underway. A panel of community members then offered their personal perspectives, leaving lots of time for audience questions.

The afternoon was a big success. Eighty per cent of participants rated the panel as "excellent," while an impressive 90 per cent gave that rating to the expert speakers.

The event also proved an effective way to reach new people. For roughly a quarter of the attendees, this was their first contact with HSC. Now, HSC is exploring the idea of hosting similar events in other areas of the country where clinical trials are taking place.



# 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.

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