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Media Contact:

Kaija Hutteri 519-749-8491 ext 130
khutteri@huntingtonsociety.ca

Silencing Huntington Disease?

Canada selected for clinical trials of drug that may halt fatal neurodegenerative illness

(KITCHENER, ON) September 10, 2014. Today, there are no treatments to slow or stop Huntington disease (HD). Tomorrow might be a different picture.

A small biotechnology company in California believes their gene-silencing approach could mean treatments for the thousands of Canadians diagnosed with this fatal, inherited neurodegenerative disease and for the thousands more at-risk of developing it.

Isis Pharmaceuticals has partnered with pharmaceutical giant Roche to launch Phase I clinical trials of a drug currently known as ISIS-HTT_{Rx}. By targeting the messenger RNA that holds instructions for making the faulty HD protein, ISIS-HTT_{Rx} attacks the root of the problem.

In 2012, Isis researchers reported that their approach not only delayed the progression of the disease in a mouse model of HD, it actually reversed the symptoms. Now they are ready to move into human trials, ultimately aiming to see whether it can achieve similar results. In spring 2015, Isis and Roche will launch Phase I clinical trials in a few selected sites in Canada and Europe.

According to Bev Heim-Myers, CEO of the Huntington Society of Canada, this is excellent news for Canadian families affected by the disease.

“The launch of Phase I trials is an extremely exciting step towards potential treatments for Huntington disease,” she says. However, she tempers her enthusiasm with several words of caution. “What works in mice may not work in humans,” she says. “Even if the drug does prove effective, it will take several years to get to market.”

Heim-Myers notes that the goal of a Phase I trial is simply to test the safety of a new drug. If ISIS-HTT_{Rx} proves safe, it then moves into phase II and phase III trials to assess how well it works. “It is a time-consuming process,” she explains.

The Society has been working to make sure trials of any potential treatment proceed as quickly as possible. For the past two years the Society has played a key role in bridging the relationship between researchers and individuals by educating Canadians on the importance of the clinical trial process; how they can get involved; and why their participation is so crucial. Partnering with Canada's Research-Based Pharmaceutical Companies (Rx&D), this initiative, called the *Be Brave, Be Bold, Be Ready: Clinical Trial Preparation* project continues to encourage doctors, scientists and people affected by HD to get involved. This strategy is groundbreaking, is carving a new path for the research community and is an opportunity for clinicians, HSC and Rx&D to partner in the outreach efforts to encourage participation in clinical trials.

With clinical trials set to begin in mid-2015, the urgency lies in not only educating as many people as possible, including efforts in rural and culturally diverse communities, but also ensuring clinicians have sustainable mechanisms in place to support the process.

“We are very proud that those efforts have paid off and that Canadian sites may be selected for Phase I trials of ISIS-HTT_{Rx}” she says. “That means if this drug proves effective, Canadians will have access to it more quickly.”

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Huntington disease (HD) is a debilitating brain disorder that is fatal and incurable. About one in every 7,000 Canadians has HD and approximately one in every 5,500 is at-risk of developing the disease. Many more are touched by HD whether as a caregiver, a family member, or a friend. HD causes cells in specific parts of the brain to die. As the disease progresses, a person with Huntington’s become less able to manage movements, recall events, make decisions and control emotions. The disease leads to incapacitation and, eventually, death.

The **Huntington Society of Canada (HSC)** is a respected leader in the worldwide effort to end Huntington disease. HSC is the only Canadian health charity dedicated to providing help and hope for families dealing with Huntington disease across Canada.

Note to editors:

Bev Heim-Myers is available for interviews:

Bev Heim-Myers
Chief Executive Officer
Huntington Society of Canada
519-749-7063