Researchers discover zip codes for protein

January 29 2007

McMaster scientists are very close to defining small molecule drugs that should be able to redirect the huntingtin protein from accumulating in the wrong place within brain cells, which could potentially translate to a therapy for Huntington's Disease (HD).

There is currently no way to stop or reverse the progression of Huntington's Disease, which affects one in 10,000 Americans. It is a progressive, and eventually fatal, genetic neurological disease.

Associate professor Ray Truant's lab has discovered molecular 'zip codes' or protein sequences in the huntingtin protein that dictate where it goes to within a brain cell.

"We have shown that the mutant huntingtin protein is mis-localized in brain cells in Huntington's Disease, because it is being improperly signaled, or instructed where to go in the cell," said Truant, of the Department of Biochemistry and Biomedical Sciences.

"In particular, Huntingtin is accumulating at the heart of the cell, the nucleus, where it shouldn't be. This is causing the brain cells to not function properly, and eventually die."

Truant and his university colleagues have received a $260,000 research operating grant from the American-based High Q Foundation. The grant will fund research using the technology of McMaster's new Biophotonics Facility and the use of laser microscopy in living brain cells.

It will also use the McMaster High Throughput Screening Facility to screen for new drugs that can affect how huntingtin is signalled.

"This class of small molecule drugs we are now working with has been proven recently to be a very successful class of drugs for different diseases, but not yet in HD," said Truant.

Source: McMaster University