



Canadian Gene Cure Foundation
Foundation Canadienne Gène Cure



The Canadian Gene Cure Foundation
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FOR IMMEDIATE RELEASE

EXCITING RESEARCH TARGETS TREATMENTS FOR RARE GENETIC DISEASES

Vancouver, BC – November 28, 2007: The Canadian Gene Cure Foundation (CGCF) is pleased to award two \$75,000 grants for research into new treatments for rare genetic diseases.

The grants have been awarded to Dr Brian Robinson, Canada Research Chair in Metabolism and Nutrition, Hospital for Sick Children, Toronto and Dr Paul Goodyer, Professor of Paediatrics, McGill University, Montreal.

Dr Robinson and his team will be testing a series of compounds to identify possible drug treatment for mitochondrial diseases. Approximately one child every week is born with a genetic abnormality, which seriously decreases the ability of their cells to produce energy. Their organs are not able to function normally, and without treatment these children suffer progressive nerve and organ failure.

Dr. Robinson said: "There are no specific cures for mitochondrial diseases. But we know that the difference between good health and neurodegenerative disease can be a matter of a few percentage points in terms of the severity of enzyme deficiency. We have identified five compounds that are known to increase mitochondrial activity. With this grant we can now test these further, with the hope that this approach will yield one or more drugs for these patients."

The second grant will enable Dr. Goodyer and his team to investigate possible new treatments for cystinosis. This rare genetic condition is ten times more prevalent in Quebec, where it is responsible for nearly one third of childhood kidney transplants. These children lack the ability to recycle the amino acid cystine, which then accumulates to toxic levels causing progressive tissue damage. Patients can be treated with cysteamine, but results are far from optimal.

Dr. Goodyer explained: 'The first part of our project will investigate a new, better-tolerated drug therapy for this disease. The second phase will investigate the use of gene therapy to treat cystine accumulation in the eye. If delivery of the normal cystinosis gene to cultured cells can normalise cystine content, this could become an important model for other eye disease.'

"The Canadian Gene Cure Foundation is delighted to provide these two grants to support such promising research into treatments for rare genetic disorders. Patients with these diseases can have extreme difficulty finding the help they need. Yet research focused on a rare genetic defect can spark therapeutic ideas applicable to many more common illnesses," said Sandra MacPherson, chair of the Canadian Gene Cure Foundation.

This year the Foundation has distributed over \$300,000 in grants to leading Canadian scientists. These grants are made possible through the donations we receive through our national public fundraiser, Jeans for Genes Day.

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About the Canadian Gene Cure Foundation:

The Canadian Gene Cure Foundation is a registered Canadian charity formed in 1999 to raise much-needed funds for medical genetics research in Canada.

For further information about the *Canadian Gene Cure Foundation*, please visit www.genecure.ca, or contact:

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