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**GROUND-BREAKING RESEARCH TARGETS  
NEW TREATMENTS FOR RARE GENETIC DISEASES**

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**Vancouver, BC – September 17, 2007:** The Canadian Gene Cure Foundation (CGCF) is pleased to award two \$65,000 grants for research into new treatments for rare genetic diseases.

The grants have been awarded to Dr Jacques Galipeau, Associate Professor, Department of Medicine and Oncology, McGill University Montreal and Dr David Rosenblatt, Chairman of the Department of Human Genetics, McGill University, Montreal.

Dr Galipeau and his team will be using their expertise in the genetic enhancement of stem cells to develop a new treatment for patients affected by the neuronopathic forms of Gaucher disease. Gaucher disease is a lysosomal storage disorder caused by a deficiency of the enzyme glucocerebrosidase. Patients typically suffer an enlargement of the liver and spleen, low blood platelets and anemia. In some patients – as many as 300 Canadians – the disease also affects the brain, resulting in a debilitating loss of motor skills. The brain dysfunction, which can prove to be fatal, is currently untreatable because enzyme supplements cannot cross the blood-brain barrier.

“We propose to use cells residing in the bone marrow to provide the missing enzyme to the brain”, explained Dr Galipeau. “The grant from CGCF will enable us to test this theory in the laboratory to see if these genetically modified cells can produce enough enzyme to have a clinical impact.”

The second grant will enable Dr. Rosenblatt and his team to explore the use of different forms of vitamin B12 to treat a rare disorder of vitamin B12 metabolism. In 2006, Dr. Rosenblatt’s team discovered a new gene (MMACHC) responsible for the most common genetic cause of vitamin B12 deficiency. Mutations to this gene prevent the body from converting vitamin B12 into its active forms. A lack of this essential nutrient leads to a variety of symptoms, from severe anemia to birth defects and developmental delay in childhood. Dr. Rosenblatt’s laboratory is one of only two in the world that is providing clinical diagnosis as well as research in this area.

“With support from the Canadian Gene Cure Foundation, we can now study how replacement of the missing vitamin with different forms of vitamin B12 may help reverse the biochemical defects in patients with different mutations, said Dr Rosenblatt. “The hope would be to use this information to determine a more rational, and ultimately more successful, approach to therapy for these patients.”

“The Canadian Gene Cure Foundation is pleased to support research into 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.

**About the Canadian Gene Cure Foundation:**

The Canadian Gene Cure Foundation is a registered Canadian charitable organization, 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](http://www.genecure.ca), or call:**

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