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GenomeCanada

Orphan Diseases: Identifying Genes and Novel Therapeutics to Enhance Treatment (IGNITE)

Status	Approved
Competition	2010 Large-Scale Applied Research Project Competition
Sector	Health
Genome Centre	Genome Atlantic
Project Leaders	Christopher McMaster and Conrad Fernandez, Dalhousie University

Project Description

Understanding the cause of genetic diseases. One in twelve Canadians suffers from an “orphan disease”. The discovery of effective treatment for these conditions is often hampered by inadequate scientific understanding of the condition, limited resource allocation to study these diseases and the cost of new drug development. While individually rare, these diseases have a cumulative socio-economic and health effect on three million Canadians. Now, gene discovery is offering new hope for new therapies. Building on the successful Atlantic Medical Genetics and Genomics Initiative, scientists are developing new therapies to orphan disease patients in a shorter time, at reduced costs. With funding from Genome Canada, researchers are working to: discover genes responsible for these diseases; locate therapeutic targets; and identify small molecules and drug leads that could lessen the impact of these diseases. Researchers are also focusing on using or converting existing drugs to more rapidly address unmet medical needs.

As part of their work, researchers are examining the ethical issues affecting orphan disease patients including evaluating international regulatory models for orphan drugs and the needs of individual recipients.

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