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Pleiades Promoter Project: Genetic Resource for CNS Regional & Cell Specific Molecular Delivery

Status	Current
Competition	III
Sector	Health
Genome Centre	Genome British Columbia
Project Leader	Elizabeth Simpson

Project Description

Gene therapy has often been heralded as a new frontier in medicine. The idea of delivering healthy genes to correct dysfunction holds promise for researchers of many diseases. But gene transfer therapy has raised several safety issues. For example, more clinically relevant research is needed. And genes need to be delivered to specific cell types and also specific locations in the genome, so that therapy does not unintentionally alter healthy cells or mutate the genome.

Dr. Elizabeth M. Simpson, Canada Research Chair in Genetics and Behaviour, and Senior Scientist, at UBC's Centre for Molecular Medicine and Therapeutics within the Child and Family Research Institute, is leading the Pleiades Promoter Project. This project is designed to use the latest scientific techniques to address these safety concerns surrounding gene therapy.

The Project's objective is to build an innovative 'tool-kit' of 160 bioinformatically-designed and biologically-validated human DNA MiniPromoters to drive gene expression in therapeutically important brain regions. Promoters are DNA sequences that regulate gene expression and determine which proteins are manufactured. MiniPromoter validation will involve inserting each promoter into a specific location in the mouse genome and visualizing gene expression in the brain. The mouse, whose genome resembles that of human, is the organism of choice for large-scale genomic manipulation.

One of the strengths of the Pleiades Project is bringing together highly qualified Canadian scientists in bioinformatics, high throughput genomics, and transgenic mouse technology to find new therapeutic approaches to brain disorders such as Alzheimer Disease, Parkinson Disease, Depression, Autism, Addiction, and Cancer.

Wide distribution and application of this research and clinical resource will be ensured by a close partnership with BioPharma Solutions, a management and communication consulting firm specializing in product development and commercialization.