Research Collaboration Opportunity

Title: Stargardt macular degeneration: Linking Drug Discovery to Therapeutic Treatments

PIs:
Robert S. Molday, Ph.D.

Therapeutic Area:
Eye diseases; retinal degenerative diseases; macular degeneration

Description:
Stargardt macular degeneration (STDG1) is a relatively common, autosomal recessive, early onset monogenic retinal degenerative disease associated with mutations in the ABC Transporter ABCA4. We have recently shown using our novel functional assays that selected pharmaceutical agents can enhance the retinal transport activity of wild-type ABCA4 and disease-causing missense mutants - demonstrating the ‘proof-of-concept’ that there are drugs that may serve as therapeutic treatments for STDG1 and related retinal diseases.

Research stage:
Preclinical studies. Expand drug discovery screening studies for STGD1 and related diseases to identify additional novel drugs for STGD1 and possibly other diseases associated with ABCA transporters; pursuit in vivo studies to demonstrate the potential application of selected drugs to diminish the disease phenotype in an animal model for STGD1. Develop the most promising drug(s) for clinical trials.

Relevant publications:

Contact
Robert S. Molday, Ph.D.
Professor
Dept of Biochemistry and Molecular Biology
2350 Health Sciences Mall
University of British Columbia
Vancouver, B.C. V6T 1Z3 Canada