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THE UNIVERSITY OF BRITISH COLUMBIA
Life Sciences Institute

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:
Quazi, F., Lenevich, S., Molday, R.S. ABCA4 is an N-retinylidene-phosphatidylethanolamine and Phosphatidylethanolamine Importer. Nat. Commun 3:925 doi: 10.1038/ncomms1927 (2012).
Quazi, F. and Molday, RS. Differential Phospholipid Substrates and Directional Transport by ATP Binding Cassette Proteins ABCA1, ABCA7, and ABCA4 and Disease-causing Mutants J Biol Chem. 288:34414-26. (2013).

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