



Centre universitaire de santé McGill
McGill University Health Centre



Patient Recruitment

Leber Congenital Amaurosis (LCA)

The Foundation Fighting Blindness of Canada (FFB-C) is proud to announce that Dr. Robert Koenekoop of McGill University is now recruiting Leber congenital amaurosis (LCA) patients with specific gene defects in RPE65 and LRAT for a drug trial to test the safety and efficacy of a new retinoid. This trial is approved by Health Canada (TPD) and McGill University Health Centre at the Montreal Children's Hospital.

This clinical trial is a Safety/Proof of Concept Study to evaluate the effects of a novel retinoid, oral QLT091001 in children with LCA. This is a phase Ib study. Patients will be treated for seven days with oral QLT091001 and evaluations will be performed to determine whether the treatment is safe, tolerable and can improve visual function in children with LCA. Up to eight children (ages 5-14 years) with LCA due to inherited deficiencies of Retinal Pigment Epithelial 65 protein (RPE65) or Lecithin: Retinol Acyltransferase (LRAT) will be treated.



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CANDIDATE PROFILE

- Boys or girls between 5 to 14 years of age diagnosed with LCA (with 2 mutation in either RPE65 or LRAT)
- The mutation (RPE65 or LRAT) must have been verified in two different laboratories (if only verified in one lab, we can organize the second)
- Subjects with a “best-corrected” ETDRS visual acuity score of 3 letters or better (20/800 Snellen equivalent)

Exclusion criteria

- Subjects who are actively participating in an experimental therapy study or who have received experimental therapy within 60 days
- Subjects with any clinically important abnormal physical finding at screening
- Subjects who have taken any prescription or investigational oral retinoid medication (e.g. Accutane, Soriatane) within 6 months of day 0 and subjects who did not tolerate their previous oral retinoid medication will be excluded regardless of the time of last exposure
- Subjects with a history of diabetes or chronic hyperlipidemia, hepatitis, pancreatitis or cirrhosis

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