Fenretinide corrects lipid imbalance in cystic fibrosis (CF) and Pseudomonas clearance from the infected CF lungs

Information Summary

Reference code: ROI 06031

Technology overview: Fenretinide, a semi-synthetic retinoid, reduces inflammation by correcting AA/DHA lipid imbalance.

Applications: Treatment of Cystic Fibrosis (CF), lung infection and osteoporosis associated with CF.

Validation: In vitro and in vivo animal data.

Inventors: Dr. Radzioch, Danuta
Dr. Kubow, Stanley

Opportunity: Exclusive or non-exclusive licence; formulation partnership

Contact: Michèle Beaulieu Ph.D. MBA
(514)398-6874
michele.beaulieu@mcgill.ca

Technology Description

Cystic Fibrosis (CF) is the most common fatal genetic disease affecting Caucasians. Patients have abnormal mucus production, pulmonary inflammation and suffer from chronic lung infections and osteoporosis. CF patients consistently exhibit imbalance of DHA and AA stemming from altered fatty acid metabolism. DHA and AA play a key role in regulating cell function, membrane fluidity, trafficking, inflammation and mucin secretion. The characteristic lipid imbalance correlates well in those organs most affected by CF, including the lungs, pancreas and intestine. Bone metabolism is also altered in CF due to abnormal intestinal function impairing calcium and vitamin D processing. The invention relates to the use of fenretinide, a semi-synthetic retinoid, to correct the defective DHA/AA ratio in the individual CF-affected organs, and specifically for its use in the treatment of lung infections and prevention of osteoporosis in CF patients. Separate patents cover the utility of fenretinide in the treatment of CF and the use of fenretinide in the treatment of Pseudomonas aeruginosa infection and prevention and treatment of CF associated osteoporosis.

Supportive Data

The invention is supported by in vitro data in Cftr-deficient cells and in vivo data, in an animal model of CF, demonstrating significantly diminished bacterial burden, decreased disease progression and reduced osteoporosis.

Benefits

Reconstituting lipid balance may constitute a novel approach to pharmacotherapy of CF and CF-associated pathologies.

Market Need and Opportunity

CF is an autosomal recessive disease with an incidence of 1 in 3,500 live births in North America and Europe. The prevalence is in the order of 30,000 in North America and 20,000 in Europe, which qualifies CF as an orphan disease. The CF market is estimated at approximately $1.25 billion due to the variety of treatments required by CF patients. Currently no effective treatments are available for CF. Fenretinide is the subject of ongoing clinical trials in multiple cancers and has shown an excellent safety profile. This could result in accelerated entry in clinical trials for other indications as well as extended patent protection.