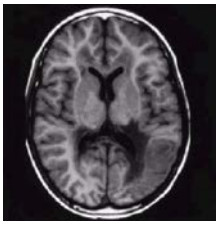
Fingolimod as new drug therapy to treat X-linked Adrenoleucodistrophy

Areas: Pharma, Medicine, Diagnostics

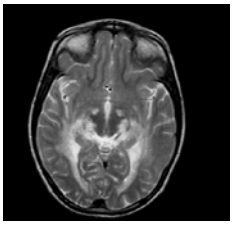
Collaboration Opportunity: Available to license (orphan drugs accelerators and/or pharma companies)

Summary: X-linked Adrenoleucodistrophy (X-ALD) is characterized by central inflammatory demyelination in the brain and/or slowly progressing axonal degeneration in the spinal cord. These neural changes are due to the loss of function of the peroxisomal transporter ABCD1 resulting in the accumulation of very-long chain fatty acids (VLCFA) that finally damage myelin surrounding nerves. As result, seizures, hyperactivity and problems for speaking, listening and understanding verbal instructions are usually detected in X-ALD patients.

A multidisciplinary team of researchers and physicians have developed **a new drug therapy and a method for the diagnosis and monitoring the progression** of patients suffering from X-ALD. Fingolimod is a marketed drug for the treatment for Multiple Sclerosis and it has also shown effectiveness for the treatment of ALD. In combination with a cocktail of selected antioxidants, the drug is capable to halter immunity attack in ALD patients and to normalize levels of sphingosine 1-phosphate, thus preventing neural demyelination. Moreover, the technology includes several biomarkers/targets for the diagnosis and monitoring of this rare disease.



Normal brain



X-ALD brain

Need: Currently there is no successful treatment for ALD although several approaches exist, including allogenic bone marrow transplantation (low rate of survival, a new clinical trial is in process); gene therapy with transfection of the ABCD2 gene (no statistical results at this moment, a new clinical trial is in process); Lorenzo's oil dietary treatment (no clinically relevant benefits) and treatments with diverse drugs such as 4-phenylbutyrate and valproic acid (not clear results), bezafibrate (no reduction of VLCFA in either plasma or lymphocytes) or sobetirome (preliminary results, in phase 1).

Market: With an incidence of 1 in 17,000 newborns, ALD is the most common monogenic leukodistrophy and peroxisomal disorder. The prevalence of X-ALD is 1 in 20,000 to 50,000 individuals worldwide and occurs primarily in males.

Commercial Applications: Fingolimod can be used as new therapy for X-ALD patients. A specific metabolite can be used to track the progression of ALD.

Competitive Advantages:

- ✓ Fingolimod is a marketed drug useful for a second use in ALD
- ✓ No effective treatment exists for ALD
- ✓ Technology ready for clinical trials at Phase IIb
- ✓ Exclusive biomarker for the development of new products or services facing diagnosis and progress monitoring

Development status: Technology demonstrated in relevant environment (TLR6) and ready for Phase II-B clinical trials.

Intellectual Property:

1. EU patent filing EP15382366.1
2. PCT filing PCT/ES2016/066825

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