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ASIA EDITION

First patient dosed in the FRAMES phase 2 trial in Friedreich's Ataxia

07 June 2019 | News

A double-blind, placebo-controlled trial aimed at assessing efficacy and safety of leriglitazone, up-regulated CNS disorders in Friedreich's Ataxia patients

Minoryx Therapeutics, a company specializing in the development of new drugs for orphan diseases, announced on 4 June 2019, that the first patient has been dosed with its lead candidate, leriglitazone (MIN102), in the phase 2 FRAMES clinical trial in Friedreich's Ataxia.

This first patient was dosed at the end of April at the Hospital La Paz (Madrid) by a team headed up by Dr Francisco Javier Rodríguez de Rivera. Additional sites, led by Prof Alexandra Durr (ICM, Paris, France), Dr Alexandra Darling (Hospital Sant Joan de Déu, Barcelona, Spain), Prof Massimo Pandolfo (Hôpital Erasme-ULB, Brussels, Belgium), and Prof Jörg Schulz (Universitätsklinikum RWTH, Aachen, Germany) are now also open for enrolment.

FRAMES is a double-blind, placebo-controlled trial with the aim of assessing the efficacy and safety of leriglitazone in Friedreich's Ataxia patients. The principal investigator is Professor Alexandra Durr from the Brain and Spine Institute of La Pitié-Salpêtrière University Hospital (ICM), Paris. The trial will enrol 36 patients aged 12 years or older with a treatment duration of one year.

Several studies have shown that the PPAR?/PGC1a pathway is down-regulated in Friedreich's Ataxia, making this pathway a therapeutic target with disease-modifying potential. In preclinical models, leriglitazone was able to up-regulate PGC1a, increase neuron survival, improve mitochondrial function and biogenesis, and restore energy production.

Leriglitazone has shown good in-vivo efficacy in other models of central nervous system (CNS) diseases and is currently in phase 2/3 clinical trial for the treatment of adrenomyeloneuropathy (AMN), the most common phenotype of X-linked Adrenoleukodystrophy (X-ALD). This trial completed enrolment of 116 patients in 2018 and about 25% of patients have now received treatment for over one year.

Marc Martinell, CEO of Minoryx, said: "We are pleased that enrolment in the FRAMES trial has started. Based on preclinical studies there is a strong rationale for developing leriglitazone in this indication and we are currently exploring a number of additional conditions affecting the central nervous system, where leriglitazone may provide potential benefit for patients."

“I’m delighted to see that Minoryx’s leriglitazone is being assessed in multiple rare CNS diseases, an area where there is a high unmet medical need for novel treatments,” said Prof. Alexandra Durr, principal investigator at the Brain and Spine Institute of La Pitié-Salpêtrière University Hospital (ICM), Paris, and coordinator of the study. “I’m looking forward to the completion and a positive outcome of this clinical trial, which could bring a long-awaited treatment option for Friedreich’s Ataxia patients.”