Minoryx Therapeutics completes enrollment in FRAMES phase 2 trial with leriglitazone in Friedreich's Ataxia

Recruitment of 39 patients in multicenter European phase 2 trial completed ahead of schedule

Mataró, Barcelona, Spain and Charleroi, Belgium, October 8, 2019 – Minoryx Therapeutics, a company specializing in the development of innovative treatments for orphan central nervous system (CNS) diseases, today announces that it has completed recruitment in the FRAMES phase 2 clinical trial of its novel PPARγ agonist, leriglitazone (MIN-102), in patients with Friedreich's Ataxia.

FRAMES is a multicenter, randomized, double-blind, placebo-controlled trial that will assess the efficacy and safety of leriglitazone in patients with Friedreich's Ataxia. Recruitment of 39 patients in four European countries was completed in just four and a half months, well ahead of schedule. The patients, aged 12-60 years, will receive leriglitazone, administered once a day as an oral suspension, for a total of one year. The primary objective of the trial is to monitor the effect on disease progression. This will be measured through state-of-the art imaging of the spinal cord. Secondary objectives include safety and tolerability, effect on additional clinical measures, such as patient reported outcomes, functional disability scores and exploratory biomarkers.

"We are very pleased with the strong support from the medical community and patients who made it possible to fully recruit FRAMES ahead of schedule, demonstrating the need for novel disease-modifying treatment options for patients suffering from this life-threatening disease," said Marc Martinell, CEO of Minoryx. "We look forward to completing treatment of all patients in this study and reporting on the data, which is expected by the end of 2020."

"We are delighted to have been able to recruit all the patients in this trial in such a short period of time," said Prof. Alexandra Durr, Brain and Spine Institute of La Pitié-Salpêtrière University Hospital (ICM), Paris, principal investigator and coordinator of FRAMES. "Friedreich's Ataxia is a devastating, orphan neurodegenerative disease which typically has an onset in patients between 5 and 18 years of age. There is a high unmet need for novel, effective disease-modifying therapies as patients currently rely on symptomatic therapies and non-pharmacological interventions to manage their disease".

Several studies have shown that the PPARγ/PGC1α pathway is downregulated in Friedreich's Ataxia (FRDA). The disease-modifying potential of leriglitazone was demonstrated in preclinical FRDA models, showing that it effectively upregulated PGC1α, increased neuron survival, improved mitochondrial function and biogenesis and restored energy production.

Leriglitazone has also proven to be effective in *in vivo* models of other CNS diseases and is currently in a pivotal phase 2/3 clinical trial for the treatment of adrenomyeloneuropathy (AMN), the most common phenotype of X-linked adrenoleukodystrophy (X-ALD). This trial completed enrollment of 116 patients, all of whom have now received treatment for over a year without experiencing any serious safety events. Results from this trial are expected to be available by the end of 2020 and the company expects to file for market authorization in Europe and the USA in 2021.

About Friedreich's Ataxia
Friedreich's Ataxia is an orphan genetic disease characterized by loss of coordination and muscle strength, resulting from the degeneration of nerve tissue in the spinal cord and damage in the nerves that control muscle movement. Symptoms range from the inability to coordinate movements to imbalance, muscle weakness and tremors. Within 10-15 years after disease onset, patients lose their ability to stand, sit and walk. Friedreich's Ataxia is fatal, mainly due to cardiac failure. Friedreich's Ataxia is caused by a genetic defect leading to frataxin deficiency. It affects approximately one in 40,000 people worldwide. There is currently no curative therapy available; existing treatments solely address symptoms.

About leriglitazone
Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPARγ agonist, that engages the target receptor within the central nervous system. It demonstrated efficacy in animal models of multiple diseases, modulating pathways leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. Leriglitazone has the potential to treat several CNS conditions, including orphan diseases, such as X-ALD and Friedreich's Ataxia. A phase 1 clinical study confirmed that leriglitazone is well tolerated and is able to cross the blood brain barrier and engage PPARγ within the central nervous system. Currently leriglitazone is being assessed in a two-year double-blind, placebo-controlled, pivotal Phase 2/3 study in adult X-ALD patients with adrenomyeloneuropathy (AMN) and in a one year double-blind, placebo-controlled Phase 2 study in patients with Friedreich's Ataxia. Results of both studies are expected by the end of 2020. Leriglitazone has received Orphan Drug Designation for the treatment of X-ALD in both the EU and the US.

About Minoryx Therapeutics
Minoryx is a clinical stage biotech company focusing on the development of novel therapies for orphan CNS diseases with high unmet medical needs. The company's lead program, leriglitazone (MIN-102), a novel, selective PPARγ agonist, is currently being evaluated in X-ALD and Friedreich's Ataxia. The company is backed by a syndicate of experienced investors and has support from a network of other organizations. Minoryx was founded in 2011, has operations in Spain and Belgium and has raised a total of €50M through Series A & B financing rounds.
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