Minoryx Therapeutics announces first patient dosed in the FRAMES phase 2 trial in Friedreich's Ataxia

Mataró, Barcelona, Spain and Charleroi, Belgium, June 4, 2019- Minoryx Therapeutics, a company specializing in the development of new drugs for orphan diseases, today announces that the first patient has been dosed with its lead candidate, leriglitazone (MIN-102), 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 enrollment.

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

Several studies have shown that the PPARγ/PGC1α pathway is downregulated in Friedreich's Ataxia, making this pathway a therapeutic target with disease modifying potential. In preclinical models leriglitazone was able to upregulate PGC1α, increase neuron survival, improve mitochondrial function and biogenesis, and restore energy production.

Leriglitazone has also shown good in-vivo efficacy in models of other central nervous system (CNS) diseases and is currently in a 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 in 2018 and about 25% of patients have now received treatment for over one year.

"We are pleased that enrollment in the FRAMES trial has started," said Marc Martinell, CEO of Minoryx. "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."

More information on the trial

About Friedreich's Ataxia
Friedreich's Ataxia is a rare 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 gait instability with imbalance, muscle weakness and tremors. Within 10 – 15 years of onset patients lose their ability to stand, sit and walk. Friedreich's Ataxia becomes fatal, mainly due to cardiac failure. Friedreich's Ataxia is caused by a genetic defect leading to frataxin deficiency which affects male and female children alike and is passed down as a recessive trait. It affects approximately 1 in 40,000 people. Currently there is no known curative therapy and existing treatments solely address symptoms.

About leriglitazone
Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPAR gamma agonist, able to engage the target receptor at the levels required for efficacy within the CNS. It showed robust preclinical proof of concept 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 was successfully completed confirming that leriglitazone is well tolerated and is able to cross the blood brain barrier and engage PPAR gamma within the CNS at an equivalent level as in preclinical studies. 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 leading the development of new therapies for orphan CNS diseases with a high unmet medical need, such as X-ALD and Friedreich's Ataxia. The company's lead program is lerigltiazone (MIN-102). The Minoryx team is made up of a group of drug discovery and development experts with several decades of experience in biotech and pharma. The company is backed by a syndicate of experienced investors and has support from a network of other organizations. Minoryx was founded in 2011 and has operations both in Spain and Belgium. It has raised a total of €50M.
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