

Minoryx's preclinical data for leriglitzone in Friedreich's Ataxia published in Neurobiology of Disease Journal

Preclinical data highlights potential therapeutic action and protection against neurodegeneration

Mataró, Barcelona, Spain and Gosselies, Belgium, November 16, 2020 - Minoryx Therapeutics, a Phase 3 clinical stage biotech company focused on the development of differentiating treatment options in orphan central nervous system (CNS) disorders with high unmet need, today announces the [publication in 'Neurobiology of disease'](#) of preclinical data on its lead compound, leriglitzone, showing potential therapeutic action and protection against neurodegeneration, in particular against Friedreich's Ataxia (FRDA).

The preclinical data, published on November 7, 2020, supports the on-going clinical development in treating FRDA. The results show that the company's lead compound, leriglitzone, improves impairments that are derived from frataxin loss – the genetic deficiency that causes FRDA.

As key highlights, the publication shows that in FRDA models, leriglitzone:

- Increases frataxin levels and mitochondrial markers
- Protects from neurodegeneration by improving mitochondrial function
- Prevents the formation of lipid droplets in frataxin-deficient cardiomyocytes
- Rescues the motor function deficit in transgenic mice
- Emerges as a potential therapeutic agent for Friedreich's Ataxia

"At Minoryx, we're committed to making new therapies available for patients suffering from severe, orphan diseases. Leriglitzone is currently in clinical development for the treatment of a range of orphan central nervous system (CNS) disorders and is currently completing a pivotal phase 2/3 clinical trial in Adrenomyeloneuropathy (AMN) and a phase 2 proof of concept trial in Friedreich's Ataxia," said Dr. Marc Martinell, co-founder and CEO of Minoryx. "This preclinical data provides further evidence of the potential for therapeutic action and protection against neurodegeneration in Friedreich's Ataxia."

About Friedreich's Ataxia

Friedreich's Ataxia (FRDA) is a devastating, orphan genetic disease characterized by loss of coordination and muscle strength, resulting from the degeneration of nerves caused by a genetic defect. The disease is characterized by frataxin deficiency leading to mitochondrial dysfunction; 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. It affects approximately one in 40,000 people worldwide. There is currently no curative therapy available; existing treatments solely address symptoms.

About leriglitzone

Leriglitzone (MIN-102) is a novel bioavailable and selective PPAR- γ agonist with a potential best-in-class profile indicated for CNS diseases. It has demonstrated sufficient brain penetration and a favorable safety profile. It showed robust preclinical proof-of-concept in animal models of multiple diseases by modulating pathways leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. Leriglitzone has successfully completed a Phase 1 clinical trial showing good safety, tolerability and CNS engagement of PPAR- γ receptors at levels equivalent to those required for efficacy in preclinical models. Leriglitzone has the potential to treat several CNS disorders, including orphan diseases, and is currently being evaluated in a registration enabling Phase 2/3 study in AMN, a registration enabling Phase 2 in cALD and in a Phase 2 in Friedreich's Ataxia.

About Minoryx

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 PPAR- γ agonist, is currently being evaluated in X-ALD and Friedreich Ataxia. The company is backed by a syndicate of experienced investors, which includes Caixa Capital Risc, Roche Venture Fund, Ysios Capital, Kurma Partners, Fund+, Chiesi Ventures, S.R.I.W, Idinvest, SFPI-FPIM, HealthEquity and Sambrinvest, and has support from a network of other organizations, including the European Investment Bank (EIB). Minoryx was founded in 2011, has operations in Spain and Belgium and has so far raised more than €85M.

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