Minoryx Therapeutics receives Orphan Drug Designation from the European Commission for leriglitazone in the treatment of Friedreich's Ataxia

Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPARγ agonist

Second Orphan Drug Designation granted by the European Commission for leriglitazone, in addition to X-linked adrenoleukodystrophy (X-ALD)

Orphan Drug Status provides a number of advantages, including clinical trial protocol assistance, scientific advice and market exclusivity

Mataró, Barcelona, Spain and Charleroi, Belgium, November 12, 2019 – Minoryx Therapeutics, a company that specializes in the development of innovative treatments for orphan Central Nervous System (CNS) diseases, today announces that its lead drug candidate, leriglitazone (MIN-102), has been granted Orphan Drug Designation by the European Commission in the treatment of Friedreich's Ataxia.

Friedreich's Ataxia is a severe, rare, genetic neurodegenerative disease characterized by loss of coordination and muscle strength. The disease results from frataxin deficiency leading to mitochondrial dysfunction. Patients today rely solely on symptomatic treatments to manage their disease. Friedreich's Ataxia affects one in 40,000 people globally and has an onset between 5 and 18 years of age.

Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPARγ agonist that engages the target receptor within the central nervous system. The disease-modifying potential and unique mode-of-action of leriglitazone have been demonstrated in multiple preclinical CNS disease models showing that it has an anti-oxidant, anti-inflammatory and neuroprotective effect. Leriglitazone improves mitochondrial function and biogenesis, promotes remyelination, ameliorates lipid metabolism and delays the progression of neurological disability. Leriglitazone is currently in late-stage clinical development in adrenomyeloneuropathy (AMN) and Friedreich's Ataxia (FRDA).

"We are very pleased that, following the recent FDA Orphan Drug Designation for leriglitazone in Friedreich's Ataxia, the European Commission has also granted Orphan Drug Status, further demonstrating the potential of this novel drug candidate and our commitment to changing the lives of patients suffering from severe orphan diseases," said Marc Martinell, CEO of Minoryx. "Leriglitazone is our lead drug candidate currently in late-stage clinical development in a number of life-threatening orphan CNS diseases. Patient enrollment has been completed in both the AMN and the FRDA studies and we are on track to present our results by the end of 2020."

About leriglitazone

Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPARγ agonist that engages the target receptor at the levels required for efficacy within the central nervous system. It has demonstrated efficacy in animal models of multiple disease modulating pathways leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. Leriglitazone has the potential to treat several CNS disorders, 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 PPARγ within the central nervous system at an

equivalent level as in preclinical studies. Leriglitazone is currently being evaluated in a two-year double-blind, placebo-controlled, pivotal Phase 2/3 study in adult X-ALD patients with adrenomyelnoeuropathy (AMN) and in a one year double-blind, placebo-controlled Phase 2 study in patients with Friedreich's Ataxia. Results from both studies are expected by the end of 2020. Leriglitazone has received Orphan Drug Designation for the treatment of X-ALD and Friedreich's Ataxia in both the EU and the US.

About Friedreich's Ataxia
Friedreich's Ataxia 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 Orphan Drug Designation
European Orphan Drug Designation is granted to medicines that aim to treat conditions that are life-threatening or chronically debilitating, and have a prevalence rate in the European Union of no more than five in 10,000. Orphan Drug Designation in Europe brings a number of benefits to drug developers, including protocol assistance, access to the centralized authorization procedure, ten years of market exclusivity upon regulatory approval of the drug and regulatory fee reductions.
www.ema.europa.eu

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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