Minoryx Therapeutics receives FDA Orphan Drug Designation for leriglitazone in Friedreich's Ataxia

Leriglitazone (MIN-102) is a novel, brain penetrant, orally bioavailable and selective PPARγ agonist
Second Orphan Drug Designation granted by the FDA for leriglitazone in addition to X-linked adrenoleukodystrophy (X-ALD)
Potential for seven years of marketing exclusivity in the USA upon approval in the orphan designation indication

Mataró, Barcelona, Spain and Charleroi, Belgium, October 17, 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 in Friedreich's Ataxia by the US Food and Drug Administration (FDA).

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 in people aged between five and 18 years old.

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 progression of neurological disability. Leriglitazone is currently in late-stage clinical development in adrenomyeloneuropathy and Friedreich's Ataxia.

"Orphan Drug Designation by the FDA for Friedreich's Ataxia is yet another important milestone for the company. It is recognition of the disease-modifying potential of leriglitazone and of our commitment to changing the lives of patients suffering from severe orphan diseases with high unmet medical needs," said Marc Martinell, CEO of Minoryx. "We recently completed enrollment in the Phase 2 study of leriglitazone in Friedreich's Ataxia and the pivotal study in patients with adrenomyeloneuropathy is progressing as planned. We are looking forward to reporting the topline data for both studies in late 2020."

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. 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 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 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 in both the EU and the US.

About Orphan Drug Designation
The Orphan Drug Designation Program of the FDA provides orphan status to drugs and biologics that are defined as those intended for the treatment, prevention or diagnosis of a rare disease or condition. This means it affects less than 200,000 people in the US or meets the cost recovery provisions of the act. Orphan designation qualifies the sponsor of the drug for the various development incentives of the Orphan Drug Act, including tax credits for qualified clinical testing. In addition, it provides seven years of marketing exclusivity upon regulatory approval of the drug in the orphan designation indication.
www.fda.gov

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 ($55.1M) through Series A & B financing rounds.
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