BioSpectrum the business of Bio & Health Sciences

Minoryx Therapeutics gets ODD for CNS disease drug

17 October 2019 | News

Minoryx Therapeutics lead drug candidate, leriglitazone (MIN-102), has been granted Orphan Drug Designation in Friedreich's Ataxia by the US FDA

Minoryx Therapeutics, a company that specializes in the development of innovative treatments for orphan Central Nervous System (CNS) diseases, has 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 PPAR gamma 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."