Minoryx Therapeutics receives US FDA fast-track designation for leriglitazone in the treatment of X-ALD

Leriglitazone (MIN-102), a novel, brain penetrant, orally bioavailable and selective PPARγ agonist, is currently in late-stage development for treatment of severe orphan CNS disorders, including X-ALD and Friedreich's Ataxia. It previously received Orphan Drug Designation from EMA and FDA for both conditions

Pivotal ADVANCE trial in adult X-ALD patients with adrenomyeloneuropathy (AMN) on track, with topline results expected by end of 2020

Mataró, Barcelona, Spain and Charleroi, Belgium, January 9, 2020 – 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 fast track designation by the US Food and Drug Administration (FDA) for the treatment of all forms of X-linked adrenoleukodystrophy (X-ALD), including adrenomyeloneuropathy (AMN) and childhood cerebral ALD (cALD).

Leriglitazone has completed enrolment in the pivotal ADVANCE trial with topline data expected in late 2020. ADVANCE is a two-year double-blind, placebo-controlled registration trial in adult X-ALD patients with adrenomyeloneuropathy (AMN). Additionally, leriglitazone has completed enrolment in the phase 2 FRAMES trial in patients with Friedreich's Ataxia.

The FDA's Fast Track program is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. A drug granted Fast Track Designation may be eligible for several benefits, including more frequent meetings and communications with the FDA and, if relevant criteria are met, the potential for Accelerated Approval and Priority Review.

"The granting of Fast Track Designation underscores the unique potential of leriglitazone to treat X-ALD, an orphan life-threatening and chronic neurodegenerative disorder for which there is currently no effective cure available," said Marc Martinell, Chief Executive Officer of Minoryx. "We are very pleased with the progress we made with the pivotal ADVANCE trial in adult patients with AMN. Enrolment was completed well ahead of schedule and patients have started to rollover into an open-label extension study. We look forward to reporting topline results by the end of this year."

María Pascual, Chief Regulatory Officer at Minoryx added, "This FDA designation comes as we prepare to report pivotal results of the ADVANCE trial at the end of 2020 and to submit a New Drug Application in the US. We are working closely with the FDA to provide the first potential disease-modifying and effective therapy for patients with AMN."

About the ADVANCE trial

The ADVANCE trial is a two year, double-blind, placebo-controlled study designed to determine the efficacy and safety of leriglitazone with an open-label extension for patients completing the double-blind part of the trial. The primary objective of the clinical trial is to evaluate the efficacy of leriglitazone on the progression of adrenomyeloneuropathy (AMN) in male patients, determined by a motor function test. A total of 116 patients have been randomized in this study in centres across Europe and the United States. Top line results are expected by the end of 2020.
https://clinicaltrials.gov/ct2/show/NCT03231878?term=minoryx&draw=2&rank=2

About the FRAMES trial
The FRAMES trial is a one year, double-blind, placebo-controlled study that assesses the efficacy and safety of leriglitazone in patients with Friedreich's Ataxia aged 12-60 years. The primary objective of the trial is to monitor the effect on disease progression measured through state-of-the art imaging of the spinal cord. A total of 39 patients have been randomized in this study in centres across Europe. Top line results are expected by the end of 2020.
https://clinicaltrials.gov/ct2/show/NCT03917225?term=minoryx&draw=2&rank=1

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 (CNS). 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 adrenomyeloneuropathy (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 X-ALD
X-ALD is the most prevalent peroxisomal disease. It is caused by mutations in the ABCD1 gene. Its estimated incidence is 1:17,000 newborns worldwide. Although it primarily affects males, heterozygous women may also develop the disease in later life. X-ALD is characterized by the accumulation of very long chain fatty acids (VLCFA), leading to a neurodegenerative disorder where the most affected tissues are the spinal cord, the brain and the adrenal cortex. The CNS-related effects lead to two main phenotypes: adrenomyeloneuropathy (AMN), characterized by progressive motor dysfunction, and cerebral ALD (cALD), characterized by severe neuroinflammation leading to early death. There is currently no pharmacological treatment available on the market. The only available alternative for cALD patients is hematopoietic stem cell transplantation (HSCT). This approach does not prevent the development of the AMN phenotype, for which there are no therapies available.

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, which includes Ysios Capital, HealthEquity, Kurma Partners, Chiesi Ventures, Roche Venture Fund, Caixa Capital Risc, Idinvest Partners, Fund+, S.R.I.W, Sambrinvest and SFPI-FPIM, 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.
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