Minoryx Phase 2/3 'ADVANCE' study results to be presented at the 2021 American Neurological Association (AAN) Annual Meeting

Additional clinical data will show benefits of leriglitazone in adrenomyeloneuropathy (AMN)

Mataró, Barcelona, Spain, April 12, 2021 - Minoryx Therapeutics, a Phase 3 clinical stage biotech company focused on the development of differentiating treatment options in orphan central nervous system (CNS) disorders, today announces that a lead investigator of the Minoryx Phase 2/3 ADVANCE clinical trial will present further results of the study at the 2021 Virtual Annual Meeting of the American Neurological Association (AAN), April 17-22, 2021.

The presentation will deliver details from the ADVANCE study that evaluated leriglitazone, a novel, brain penetrant and selective PPAR γ agonist, in male patients with adrenomyeloneuropathy (AMN). AMN is a neurodegenerative disease that causes progressive myelopathy leading to spastic paraparesis and autonomic nervous system dysfunction. About 60 percent of AMN patients also develop cerebral lesion progression leading to cALD, a devastating form of the disease rapidly leading to severe disability, and where patients have an average survival of three years from onset.

In addition to the topline results released on January 26, 2021, the presentation will include previously undisclosed details on the clinical outcomes, safety and the effects on biochemical biomarkers in plasma. Overall results support the positive effects of leriglitazone on reducing the progression of cerebral lesions and myelopathy symptoms.

The presentation will be given by Reza Seyedsadjadi, MD, assistant professor of neurology at Harvard Medical School, and director of the Charcot-Marie-Tooth Center of Excellence at the Massachusetts General Hospital. Dr. Seyedsadjadi was selected to present on behalf of the ADVANCE investigator group for his substantial role in conducting the ADVANCE study at the clinical site in Boston, a world-leading expert center in AMN. The presentation will be during the Clinical Trials Session, on Tuesday, April 20, 2021, from 10:00am to 12:30pm (US Eastern time), and is entitled: '*Leriglitazone improved progression of myelopathy-related symptoms, and reduced cerebral lesions in patients with adrenomyeloneuropathy in a phase 2/3 clinical study'.* To view the presentation virtually, registration for AAN can be found here.

The presentation was accepted in the context of AAN's Emerging Science program that highlights the most current research being done in the field of neurology. This part of the conference is reserved for global research results obtained after the AAN Annual Meeting general abstract submission. The presentation needs to 'have sufficient scientific importance to warrant expedited presentation and publication'. AAN's scientific committee accepted the presentation as *'a critical advance in the field of neuroscience and worthy of presentation during this session.'*

"Minoryx's Phase 2/3 ADVANCE study is the first large clinical trial in AMN with protocol input from major global regulators. This AMN trial is vital for patients suffering from a high unmet medical need and with no currently approved treatment," said Uwe Meya, CMO, Minoryx. *"On behalf of the ADVANCE global investigator group we submitted this presentation to present these results at AAN's Annual Meeting as a conference for a leading neurological scientific*

society with delegates from the US as well as across the globe. This enables the international neurological community to discuss Minoryx's approach for a variety of orphan diseases with no available therapeutic options and leriglitazone's significant clinical benefits in patients suffering from AMN. A second clinical study that investigates the effects of leriglitazone on the progression of cerebral lesions and clinical symptoms in boys aged 2 – 12 years is currently ongoing."

Leriglitazone has been granted orphan drug status *for X-linked adrenoleukodystrophy* from the FDA and the EMA and fast track and rare pediatric disease designation from the FDA for the treatment of X-ALD. Following these clinical results Minoryx is completing documentation to be submitted to regulatory authorities for discussions for approval of leriglitazone for AMN patients.

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 PPARγ agonist, is currently being evaluated in X-linked Adrenoleukodystrophy (X-ALD) and Friedreich's 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. Minoryx was founded in 2011, is headquartered in Spain with Belgian facilities and has so far raised more than €85M.
http://www.minoryx.com

About leriglitazone
Leriglitazone (MIN-102) is Minoryx's novel orally bioavailable and selective PPARγ agonist with a potential first-in-class and best-in-class profile indicated for CNS diseases. The brain-penetrating molecule has demonstrated a favorable safety profile and 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.

About X-ALD
X-ALD (X-linked adrenoleukodystrophy) is an orphan neurodegenerative disease. The global incidence of X-ALD is approximately 6.2/100,000 live births and AMN and cALD are the two most common phenotypes. AMN affects all patients reaching adulthood and is characterized by progressive spastic paraparesis, sensory dysfunction and incontinence. This form progresses chronically with onset of symptoms typically in adulthood, affecting both men and women and has poor prognosis. cALD typically affects boys with an age of onset between 4-8 years, although recent literature suggests that up to 60% of adult AMN patients develop cALD in an average time of 10 years since onset of myelopathy. Untreated cALD patients progress quickly, as severe neurological function impairment appears 6-24 months after disease onset, often leading to permanent disability and death within 2-4 years. There is currently no approved treatment available for AMN. The only available treatment for cALD is hematopoietic stem cell transplantation (HSCT). However, HSCT is a very aggressive procedure and there is no evidence that prevents patients from progressing to AMN later in their lives.

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