Additional data from Minoryx's Phase 2/3 ADVANCE clinical trial presented at American Neurological Association (ANA) 2021

Presentation highlights reduction in cerebral lesion progression and the risk of developing cerebral ALD in AMN patients based on MRI and plasma biomarker data.

Mataró, Barcelona, Spain, November 3, 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 additional data of its Phase 2/3 ADVANCE clinical trial has been presented in a poster presentation by Dr. Fanny Mochel, Associate Professor at Sorbonne University, at the 146th Annual Meeting of the American Neurological Association (ANA) 2021.

Minoryx's Phase 2/3 ADVANCE clinical trial was a pivotal multicenter, double-blind and placebo-controlled study conducted in the United States and Europe in adult male patients with AMN. The results showed that Minoryx's therapy leriglitazone reduces the progression of cerebral lesions and, based on plasma biomarkers, modulates neuroinflammation, preserves blood-brain barrier integrity and protects against axonal degeneration. In addition, leriglitazone showed a reduction in the risk of developing progressive cerebral ALD (cALD). These data also support Minoryx's ongoing NEXUS study, an open-label phase 2 trial assessing leriglitazone in male pediatric patients with early stage cALD.

In the ADVANCE trial, of 116 patients randomized, 77 received leriglitazone and 39 received placebo. Six patients (15.4%) in the placebo group clinically developed progressive cALD compared with no patient (0%) in the leriglitazone group. Plasma biomarker data showed that neurofilament light levels were significantly increased at week 96 in placebo patients with cerebral lesion progression, supportive of a drug effect on axonal degeneration. Treatment with leriglitazone also significantly reduced plasma levels of MMP-9 a marker of blood-brain barrier integrity, and reduced or stabilized plasma levels of inflammatory biomarkers such as MIP-1β, IL-18 and IL-1ra. Furthermore, while at baseline both placebo and leriglitazone groups were well balanced in terms of number of patients with Loes severity score greater than 0, increase in this score was significantly greater in the placebo group.

X-ALD is an orphan inherited neurodegenerative disease. The disease results in very long-chain fatty acids accumulating in plasma and tissues, including the brain, spinal cord and adrenal cortex. The most common form of X-ALD is AMN, which is a chronic disease affecting all male and female X-ALD patients reaching adulthood. There is currently no approved treatment for these patients.

X-ALD patients can also develop an acute form, cerebral ALD (cALD). This results in brain inflammation leading to permanent disability and death within 2-4 years. cALD typically affects boys with an age of onset between 4-8 years. However, up to 60% of adult males with AMN can also develop this aggressive phenotype. For cALD the only currently available treatments are based on Hematopoietic stem cell transplantation (HSCT), but in adult patients this procedure is associated with significant risks and many AMN patients are not eligible for it.

"AMN is a condition with a high unmet medical need with no currently approved treatment. Minoryx's Phase 2/3 ADVANCE study is the first international and robust study providing evidence of drug effect in this population," said Marc Martinell, CEO, Minoryx. *"Additional*

analysis of the ADVANCE study data has shown that leriglitazone may provide significant clinical benefits in multiple endpoints related with cerebral lesion progression"

"There is an urgent need for therapies to treat and prevent cALD, particularly in the adult AMN population where hematopoietic stem cell transplantation has significant limitations," said Dr. Fanny Mochel *"Reducing the risk of developing progressive cALD is vital and holds promise for patients suffering from AMN who currently have no available therapeutic options."*

Leriglitazone has been granted orphan drug status for X-ALD from the FDA and the EMA and fast track and rare pediatric disease designation from the FDA for the treatment of X-ALD. Minoryx is currently under discussions with regulatory authorities for an approval path 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 €85 million. 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. It has demonstrated sufficient brain penetration and a favorable safety profile. It showed 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. In clinical trials, leriglitazone showed clinical benefit both for X-ALD and Friedreich's Ataxia patients.

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 treatments for cALD are based on hematopoietic stem cell transplantation (HSCT). However, HSCT is an aggressive procedure, it cannot be used in AMN patients with advanced myelopathy and there is no evidence that it prevents patients from progressing to AMN later in their lives.

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