Minoryx Therapeutics and Sperogenix Therapeutics enter into an exclusive license agreement to develop and commercialize leriglitazone in mainland China, Hong Kong and Macau

- **Sperogenix will receive exclusive rights to develop and commercialize leriglitazone for the treatment of X-linked adrenoleukodystrophy (X-ALD), a rare life-threatening neurological condition**
- **Minoryx will receive an upfront and milestone payments of up to $78 million, as well as double digit royalties on annual net sales**

Hong Kong, Beijing, Shanghai (China) and Mataró, Barcelona (Spain), September 23, 2020 – Sperogenix Therapeutics, a platform company dedicated to developing and commercializing rare disease therapeutics in China, and Minoryx Therapeutics, a company that specializes in the development of innovative treatments for orphan Central Nervous System (CNS) diseases, today announce that they have entered into an exclusive license agreement for the development and commercialization of leriglitazone, Minoryx's brain penetrating disease-modifying PPAR-γ agonist.

Under the terms of the agreement, Sperogenix will receive exclusive rights to develop and commercialize leriglitazone in mainland China, Hong Kong special administrative region (SAR) and Macau SAR, for the treatment of X-linked adrenoleukodystrophy (X-ALD), a life-threatening orphan neurological condition. Minoryx will receive an initial upfront payment and pre-defined regulatory and commercial milestone payments of up to $78 million , as well as double-digit royalties on annual net sales.

Leriglitazone is a novel bioavailable and selective PPAR-γ agonist, with the potential to become the world's first treatment for X-ALD. It has received Orphan Drug Designation for X-ALD in both the U.S. and Europe, and Fast Track Designation as well as Rare Pediatric Disease Designation from the U.S. FDA. A registration enabling trial in adult X-ALD patients with adrenomyeloneuropathy (AMN) is currently ongoing in the EU and in the U.S. (ADVANCE trial). It is a global multi-centric, double-blind, placebo-controlled Phase 2/3 study with data expected by the end of 2020. In addition, leriglitazone is being evaluated in a registration enabling open-label Phase 2 study in pediatric patients with cerebral X-ALD (cALD) in Europe, with topline results anticipated by mid-2021.

"This exclusive license agreement entered for leriglitazone further strengthens Sperogenix's pipeline in neurological rare diseases," said Mr. Alan (Zhiyu) Yan, co-founder, chairman and CEO of Sperogenix. "It demonstrates our strong dedication and long-term commitment to address the huge unmet medical needs in the rare disease field in China. We look forward to collaborating with all stakeholders to bring this therapy to Chinese patients as early as possible."

"We are very pleased to enter into this exclusive license agreement with Sperogenix based on its deep insights in rare diseases in China and its unique capabilities, which make us highly confident in our partnership," said Dr. Marc Martinell, co-founder and CEO of Minoryx. "This is a major milestone that underscores the potential of leriglitazone to address an important unmet medical need."

About Sperogenix Therapeutics
Sperogenix Therapeutics is a platform company dedicated to developing and commercializing rare disease therapeutics in China with focus on mid-to-late clinical stage and commercial stage products. With prioritized therapeutic areas, such as Pulmonary Vascular Disorders, Neurological Disorders, Inherited Metabolic Diseases, and Non-oncology Hematology Disorders, Sperogenix is dedicated to establishing an innovative commercial model tailored to the China rare disease field, in order to provide affordable and reliable products and services to Chinese physicians and patients. Sperogenix was founded in 2019 and is backed by biopharma industry blue chip investors including Lilly Asia Ventures (LAV) and Morningside Venture in the A-round financing.
www.sperogenix.com

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 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, has operations in Spain and Belgium and has so far raised more than €50M.
www.minoryx.com

About leriglitazone
Leriglitazone (MIN-102) is a novel bioavailable and selective PPAR-γ agonist with a potential best-in-class profile indicated for CNS diseases. It has a demonstrated sufficient brain penetration and 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. Leriglitazone has successfully completed a phase 1 clinical trial showing good safety, tolerability and CNS engagement of PPAR-γ receptors at levels equivalent to those required for efficacy in preclinical models. Leriglitazone has the potential to treat several CNS disorders, including orphan diseases, and is currently being evaluated in a registration enabling Phase 2/3 study in AMN, a registration enabling Phase 2 in cALD and in a Phase 2 in Friedreich's Ataxia.

About X-ALD
X-ALD (X-linked adrenoleukodystrophy) is an orphan neurodegenerative disease. AMN and cALD are the two most common phenotypes of X-ALD, which account for 45% and 35% respectively. The global incidence of X-ALD is approximately 6.2/100,000 live births. X-ALD is included in the first China Rare Disease Catalogue published in 2018.
The age of onset of cALD patients is typically 4-8 years old. Untreated patients progress quickly, as severe neurological function impairment appears 6-24 months after disease onset, leading to early death in 2-4 years.
AMN is characterized by progressive spastic paraparesis, sensory dysfunction and incontinence. This form progresses chronically with onset of symptoms typically in adulthood and poor prognosis.
There is currently no therapeutic treatment available for X-ALD. Several observational studies have demonstrated that hematopoietic stem cell transplantation (HSCT) may improve the five-year overall survival for cALD patients. However, there is no evidence that HSCT improves clinical outcomes of patients with AMN.

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