Press Release
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Minoryx's marketing authorization application for its lead candidate leriglitazone validated by EMA for orphan indication X-linked adrenoleukodystrophy (X-ALD)

Mataró, Barcelona, Spain, September 14, 2022 - **Minoryx Therapeutics**, a late-stage biotech company focused on the development of treatments for orphan central nervous system (CNS) disorders, today announces that it has filed a Marketing Authorization Application (MAA) for its lead candidate leriglitazone to the European Medicines Agency (EMA) for the treatment of adult male patients with X-linked adrenoleukodystrophy (X-ALD). The EMA has now validated the MAA file and it is under review by the Committee for Medicinal Products for Human Use (CHMP).

Minoryx's leriglitazone is a novel, brain penetrant and selective PPAR gamma agonist. The MAA is based on data from Minoryx's pivotal ADVANCE study, a double-blind, placebo-controlled study conducted in Europe and the United States in 116 patients. ADVANCE is the first large and definitive study to target adult male patients with X-ALD. In ADVANCE, leriglitazone reduced the progression of cerebral lesions and incidence of cALD, as well as the progression of myelopathy symptoms, such as balance deterioration. These data are supported by the ongoing open label extension of ADVANCE and NEXUS, an open-label registration trial assessing leriglitazone in boys with cALD. Results from the ADVANCE study were presented at the American Neurological Association (AAN) Annual Meeting 2021.

X-ALD is an orphan, inherited neurodegenerative disease. X-ALD patients reaching adulthood develop adrenomyeloneuropathy, which is a highly debilitating chronic form of the disease. The greatest risk to X-ALD patients, both pediatric and adult, is the development of the acute cerebral form, cALD, that leads to aggressive brain inflammation, and permanent disability and death within 2-4 years.

"The recent EUR 56 million funding and this submission are two major milestones in Minoryx's preparations towards approval and commercialization of leriglitazone in X-ALD in the EU, as well as the further development of the therapy," said Marc Martinell, CEO, Minoryx. "Leriglitazone, if approved, would be the very first therapeutic option for adult X-ALD patients with this devastating orphan disease with a major unmet medical need. During the MAA review by EMA, Minoryx will continue to investigate the benefits of leriglitazone in further X-ALD patient populations."

"The initiation of the MAA review process by the EMA brings Minoryx one step closer to providing a treatment for patients and families living with this rare devastating disorder," said Maria Pascual, Chief Regulatory Officer of Minoryx. "We will continue to work closely with the EMA through the evaluation process to maximize the chance for this therapy to reach patients in desperate need."

Minoryx is currently in discussions with the FDA to define the next steps for its US approval path for leriglitazone. Leriglitazone has been granted orphan drug status from the FDA and the EMA and fast track and rare pediatric disease designation from the FDA for the treatment of X-ALD.

About Minoryx
Minoryx is a registration 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, brain penetrant and selective PPAR gamma agonist, is being developed in X-linked Adrenoleukodystrophy (X-ALD) and other orphan CNS diseases. The company is backed by a syndicate of experienced investors, which includes Columbus Venture Partners, CDTI Innvierte, Caixa Capital Risc, Fund+, Ysios Capital, Roche Venture Fund, Kurma Partners, Chiesi Ventures, S.R.I.W, Idinvest Partners / Eurazeo, SFPI-FPIM, HealthEquity, Sambrinvest and Herrecha, 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 EUR 115 million.

About X-ALD

X-ALD (X-linked adrenoleukodystrophy) is an orphan neurodegenerative disease. The global incidence of X-ALD is approximately 6-8/100,000 live births. All X-ALD patients reaching adulthood develop adrenomyeloneuropathy (AMN), characterized by progressive spastic paraparesis, as well as progressive deterioration of balance and sensory function, and development of 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, but recent literature indicates that up to 60 percent of adult X-ALD patients will also develop cALD. cALD is characterized by aggressive brain inflammation, and if untreated, patients progress quickly with severe neurological impairment, often leading to permanent disability and death within 2-4 years. There is currently no pharmacological treatment available for X-ALD. In childhood, hematopoietic stem cell transplantation (HSCT) can arrest the disease, however, it is an aggressive procedure and only available for a portion of patients. In adults, experience in HSCT is very limited and the intervention is often not recommended.