PRESS RELEASE

Update on Regulatory Review of leriglitazone in the EU

A re-examination for cerebral adrenoleukodystrophy (cALD) will be requested following the negative CHMP opinion on X-linked adrenoleukodystrophy (X-ALD)

Barcelona, Spain and Düsseldorf, Germany – 26 January, 2024 – Minoryx Therapeutics, a late stage biotech company focused on the development of therapies for orphan central nervous system (CNS) disorders and Neuraxpharm Group (Neuraxpharm), a leading European specialty pharmaceutical company focused on the treatment of central nervous system (CNS) disorders, today announce that the EMA's Committee for Medicinal Products for Human Use (CHMP) has recommended not to grant marketing authorization for leriglitazone as a treatment of X-ALD.

Minoryx and Neuraxpharm are seeking a re-examination for conditional approval for patients with cerebral adrenoleukodystrophy (cALD), which is characterized by demyelinating brain lesions which can become rapidly progressive, leading to acute neurological decline and death in three to four years.

Minoryx and Neuraxpharm strongly believe that leriglitazone has a positive benefit / risk balance in patients with cALD. Data from ADVANCE[1], in adult male patients with ALD, showed that leriglitazone reduces the progression of lesions and the development of progressive cALD. This is now supported by 24 week data from NEXUS[2], in male pediatric patients with early stage cALD, showing a reduction in lesion progression comparable to Hematopoietic Stem Cell Transplantation (HSCT)-based approaches. These findings are being validated through confirmatory long term follow-up from ongoing trials NEXUS and CALYX[3], in adult patients with progressive cALD. The companies believe that leriglitazone could be a lifesaving treatment for these patients in a disease without pharmacological treatment options available. This unmet need underscores the critical and pressing nature of this appeal.

Marc Martinell, CEO, Minoryx said: "*We are obviously disappointed with the Committee's decision, but we strongly believe that leriglitazone provides clinically meaningful benefits for patients by reducing the development of progressive cALD and stabilizing lesion progression. Consequently, we are going to request re-examination of leriglitazone for conditional marketing authorisation for treatment of patients with cALD. Furthermore, we continue to generate additional evidence from the two ongoing confirmatory trials (CALYX and NEXUS) and we remain committed to bringing this new therapeutic option to the broader ALD patient community. We are enormously grateful for their continued support.*"

Dr. Jörg-Thomas Dierks, CEO, Neuraxpharm said: "*Leriglitazone has the potential to transform the lives of those suffering from cALD. In partnership with Minoryx. we remain committed to obtaining approval for leriglitazone as we strongly believe that these patients and their families deserve a broadly accessible and effective treatment option. This is a cruel and fast-moving disease which is devastating for patients and their families. In such an acute and fatal disease, with no other treatment options, this therapy could be critically important for patients.*"

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About leriglitazone

Leriglitazone is Minoryx Therapeutics's novel orally bioavailable and selective PPAR gamma agonist with a potential first-in-class and best-in-class profile for CNS diseases. It has demonstrated 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 in both adult X-ALD patients in ADVANCE and pediatric X-ALD patients in NEXUS. Data from ADVANCE showed that leriglitazone reduces the progression of lesions and the development of progressive cALD. Results on radiological stabilization seen in NEXUS after 24 weeks of treatment were similar to those attained with Hematopoietic Stem Cell Transplant (HSCT) or ex-vivo gene therapy, hence it is expected that leriglitazone could provide a comparable clinical benefit to cALD patients. 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.

About X-ALD and cALD

X-linked adrenoleukodystrophy (X-ALD) is an orphan neurodegenerative disease. The global incidence of X-ALD is approximately 6-8/100,000 live births. Boys and adult men with X-ALD can, at any point in their lifetime, develop cALD, which is characterized by demyelinating brain lesions that may become rapidly progressive, leading to acute neurological decline and death. These lesions can produce severe symptoms such as loss of voluntary movements, inability to swallow, loss of communication, cortical blindness and total incontinence and death with a mean survival of 3 to 4 years.

Progressive cALD occurs in 31-35% of ALD patients in childhood with typical onset between the age of 2-12 and up to 60% of adult patients, with X-ALD will develop progressive cALD over time. There is currently no pharmacological treatment available for cALD. 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; autologous HSCT is not globally available; this still requires hemo-

ablation therapy with associated comorbidities and long-term safety data is being gathered. In adults, experience in HSCT is very limited and the intervention is often not recommended.

In addition, 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.

About the Neuraxpharm Group

Neuraxpharm is a leading European specialty pharmaceutical company focused on the treatment of the central nervous system (CNS), including both psychiatric and neurological disorders. It has a unique understanding of the CNS market built over 35 years.

Neuraxpharm is constantly innovating, with new products and solutions to address unmet patient needs and is expanding its portfolio through its pipeline, partnerships and acquisitions.

The company has more than 1,000 employees and develops and commercializes CNS products through a direct presence in more than 20 countries in Europe, two in Latin America, and globally via partners in more than 40 countries. Neuraxpharm is backed by funds advised by Permira.

Neuraxpharm manufactures many of its pharmaceutical products at Neuraxpharm Pharmaceuticals (formerly Laboratorios Lesvi) in Spain.

For more information, please visit www.neuraxpharm.com

About Minoryx

Minoryx Therapeutics is a registration stage biotech company focusing on the development of novel therapies for orphan central nervous system (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 to treat 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 €120 million.

For more information, please visit https://www.minoryx.com/.

[1] ADVANCE, a pivotal phase 2/3 randomized, double-blind, placebo-controlled, clinical study with an open-label extension, was designed to assess the efficacy and safety of leriglitazone in male patients with X-ALD.
[2] NEXUS, a phase 3, open-label clinical study that designed to assess the efficacy and safety of leriglitazone in male pediatric patients with early stage cALD.
[3] CALYX, a phase 3, multicenter, randomized (1:1), double-blind, placebo-controlled, clinical study, has been designed to compare the efficacy and safety of leriglitazone in male adult patients with progressive cALD.