Update on Regulatory Review of NEZGLYAL® (leriglitazone) in the EU

Despite CHMP upholding the negative opinion for treatment of cerebral adrenoleukodystrophy (cALD) the companies remain committed to the continued development of NEZGLYAL® for cALD

Barcelona, Spain and Düsseldorf, Germany – 31 May, 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 CNS disorders, today announced that the EMA's Committee for Medicinal Products for Human Use (CHMP) has recommended not to grant marketing authorization for NEZGLYAL® (leriglitazone) as a treatment for cALD following completion of the re-examination procedure.

Minoryx and Neuraxpharm had sought re-examination for conditional marketing approval for patients with cerebral adrenoleukodystrophy (cALD), a devastating rare disease which is characterized by demyelinating brain lesions that can become rapidly progressive, leading to acute neurological decline and death in three to four years. The companies continue to believe that NEZGLYAL® could be a lifesaving treatment in a disease that has no available pharmacological treatment options.

Marc Martinell, CEO of Minoryx said: "*NEZGLYAL® has shown a clinically relevant effect in both adults and children with cALD, and we are very disappointed that the Committee considered the benefit insufficiently established, which is different from the views expressed by the CHMP's Neurology Scientific Advisory Group (SAG-N). We strive to have NEZGLYAL® approved as soon as possible and we continue to generate additional evidence from the two ongoing confirmatory trials (CALYX and NEXUS). We remain committed to bringing this new therapeutic option to the ALD patients.*"

Sara Hunt, CEO of Alex TLC, The Leukodystrophy Charity said: *"Having access to an approved and effective treatment for cALD is critical for the ALD community. This disease continues to devastate the lives of patients and their families, and it is therefore very incredibly disheartening that NEZGLYAL® was not recommended by the CHMP at this stage, especially as there are no other treatment options for this patient cohort. We sincerely hope that the development of NEZGLYAL® can continue and satisfy the CHMP procedures at the earliest opportunity."*

Dr. Jörg-Thomas Dierks, CEO of Neuraxpharm said: "*Patients desperately need treatment for this terrible and unremitting disease, and it is frustrating that we cannot swiftly make NEZGLYAL available to the patients that suffer from this life-threatening condition. We remain confident in the therapeutic potential of NEZGLYAL and expect to support Minoryx in this ongoing process. We believe that the drug will ultimately secure approval and be able to bring hope to patients.*"

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About NEZGLYAL® (leriglitazone)

NEZGLYAL® (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, NEZGLYAL® showed clinical benefit in both adult X-ALD patients in ADVANCE and pediatric X-ALD patients in NEXUS. Data from ADVANCE showed that NEZGLYAL® 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 NEZGLYAL® could provide a comparable clinical benefit to cALD patients. NEZGLYAL® 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. In Europe, NEZGLYAL® is exclusively licensed to Neuraxpharm.

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. 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 c. 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 50 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 https://www.neuraxpharm.com

About Minoryx

Minoryx Therapeutics is a late-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, NEZGLYAL® (leriglitazone), 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 adult male patients with X-ALD.
[2] NEXUS, a phase 2/3, 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 assess the efficacy and safety of leriglitazone in male adult patients with progressive cALD.