Minoryx Therapeutics receives European Orphan Drug Designation for its lead candidate MIN-102

MIN-102 targets X-linked Adrenoleukodystrophy (X-ALD), a life threatening orphan CNS disease with high unmet medical need

Orphan Drug Designation by the European agency brings a number of benefits to Minoryx including ten years market exclusivity

Mataró, Barcelona, Spain, December 14, 2016 – Minoryx Therapeutics, a drug development company specialized in the discovery and development of new drugs for orphan diseases, today announces that its lead compound MIN-102 has been granted Orphan Drug Designation by the European Medicines Agency (EMA).

MIN-102 is a selective PPAR gamma agonist with a superior profile for central nervous system related diseases. It has shown robust preclinical proof of concept in multiple animal models. Phase I studies were initiated based on these results. A phase 2/3 trial in adult AMN patients will be launched during the first half of 2017.

Minoryx Therapeutics' MIN-102 targets X-linked adrenoleukodystrophy (X-ALD), a rare and chronically debilitating life threatening neurodegenerative disease. Currently, there are no pharmacological treatments for X-ALD. MIN-102 is the only product in development for potential use across all the main phenotypes.

"We are pleased to have received European Orphan Drug Designation for MIN-102. This represents an important milestone," said Marc Martinell, CEO of Minoryx. "We are committed to progressing this drug candidate rapidly with the aim of providing a pharmacological treatment option for caregivers and for X-ALD patients."

European Orphan Drug Designation is granted to medicines that are aimed at a condition that is life-threatening or chronically debilitating and have a prevalence rate in the European Union of no more than five in 10,000.

Orphan Drug Designation by the European Medicines Agency brings a number of benefits to drug developers, including protocol assistance, prioritized scientific advice, ten years of market exclusivity after marketing the drug and potential regulatory fee reductions.

About MIN-102
MIN-102 is a novel, orally bioavailable and selective PPAR gamma agonist. It is a metabolite from pioglitazone. MIN-102 showed a superior brain penetration and

safety profile, allowing PPAR gamma engagement above the level that can be safely achieved with pioglitazone and other glitazones. It showed robust preclinical proof of concept in several animal models. In X-ALD, mutations on ABCD1 trigger a cascade of events leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. MIN-102, through its PPAR gamma activity, prevents such dysfunctions, thus it has the potential to treat both adrenomyeloneuropathy (AMN) and cerebral ALD (cALD). Phase 1 results are expected by Q1 2017. A phase 2/3 trial in adult AMN patients is planned by the first half of 2017.

About X-ALD

X-ALD is the most prevalent peroxisomal disease. It is caused by mutations on the ABCD1 gene. Its estimated incidence is 1:17,000 newborns. Although it primarily affects males, heterozygous women also develop the disease later in life. X-ALD is characterized by the accumulation of very long chain fatty acids (VLCFA) leading to a neurodegenerative disorder where the most affected tissues are the spinal cord, the brain and the adrenal cortex. The CNS related effects lead to two main phenotypes: adrenomyeloneuropathy (AMN), characterized by progressive motor dysfunction, and cerebral ALD (cALD), characterized by severe neuroinflammation leading to early death. There is currently no pharmacological treatment available on the market. The only available alternative for cALD patients is hematopoietic stem cell transplantation (HSCT). This approach does not prevent the development of the AMN phenotype, for which there are no therapies available.

About Minoryx Therapeutics

Minoryx is a clinical stage biotech company leading the development of new therapies for X-ALD and other inborn errors of metabolism, a group of rare diseases of genetic origin with a high unmet medical need. The company's leading program, now in phase 1 clinical trials, is a differentiated PPAR gamma agonist (MIN-102) that has multiple CNS indications. Minoryx harnesses its unique mechanism of action for potential use in X-ALD, a genetic disease characterized by progressive neurologic deterioration with no available pharmacological treatment. Minoryx is also working on a new class of compounds; non-competitive pharmacological chaperones, identified through its innovative proprietary platform – SEE-Tx. The Minoryx team is made up of a group of drug discovery and development experts with several decades of experience in biotech and pharma. The company is backed by a syndicate of experienced investors and has support from a network of other organizations. Minoryx was founded in 2011 and has raised a total of €24.4M.

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