MINORYX THERAPEUTICS RECEIVES ORPHAN DRUG DESIGNATION FROM THE US FDA FOR ITS LEAD CANDIDATE MIN-102

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

Orphan drug status for MIN-102 has now been granted by both the FDA and the EMA.

Mataró, Barcelona, Spain, February 22, 2017 – 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 US Food and Drug Administration body (FDA).

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.

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

There are two main clinical phenotypes of X-ALD: adrenomyeloneuropathy (AMN), characterized by progressive motor dysfunction, and inflammatory cerebral ALD (cALD), characterized by severe neuroinflammation leading to early death. A phase 2/3 trial in adult AMN patients will be launched during the first half of 2017.

“We are delighted that our lead candidate, MIN-102, now has Orphan Drug Designation from both the FDA and the EMA,” said Marc Martinell, CEO of Minoryx. “These acknowledgements prove that our drug candidate addresses an unmet need in orphan diseases. We are committed to progressing it rapidly through the next phases of drug development in order to offer a pharmacological treatment for X-ALD.”

To receive the FDA Orphan Drug status, a drug must be aimed at a rare disease or at a condition that affects less than 200,000 people in the United States. Orphan Drug Designation by the FDA grants seven years of market exclusivity in the US and has other benefits such as tax credits, protocol assistance and research grants.

At the end of 2016, MIN-102 received Orphan Drug Designation from the European Medicines Agency (EMA), guaranteeing ten years of market exclusivity in the European markets, among other benefits.

About MIN-102

MIN-102 is a novel, orally bioavailable and selective PPAR gamma agonist. It is a metabolite of pioglitazone. MIN-102 shows 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 in 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 lead program, now in phase I clinical trials, is a differentiated PPAR gamma agonist (MIN-102) that has potential in multiple CNS indications. MIN-102 has a unique mechanism of action for 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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