

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

22 February 2017 | News

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



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.