# Minoryx Therapeutics announces early completion of patient randomization in the ADVANCE trial: a phase 2/3 clinical study of MIN-102 in X-ALD patients

**Mataró, Barcelona, Spain, December 12, 2018**- Minoryx Therapeutics, a company specializing in the development of new drugs for orphan diseases, today announces that it has completed patient randomization of its phase 2/3 clinical trial of MIN-102 for the treatment of adrenomyeloneuropathy (AMN). The trial enrolled adult male patients affected by AMN, the most frequent phenotype of X-linked adrenoleukodystrophy (X-ALD).

The ADVANCE trial is a randomized, double-blind, placebo-controlled study designed to determine the efficacy and safety of MIN-102 with an open-label extension for patients completing the double-blind part of the trial. The primary objective of the clinical trial is to evaluate the efficacy of MIN-102 on the progression of adrenomyeloneuropathy (AMN) in male patients, determined by a motor function test. Results of the study are expected at the end of 2020.

A total of 116 patients have been randomized in this study. 90 patients are being treated in Europe in a number of centers in Spain, France, Hungary, Germany, Italy, the United Kingdom and the Netherlands, while 26 are in the US in California, Maryland and Massachusetts. Enrolment was completed several months ahead of schedule.

"The strong interest in this AMN trial in the EU and US has resulted in exceeding the target enrolment ahead of schedule," said Dr. Uwe Meya, chief medical officer of Minoryx. "We believe this highlights the very high unmet medical need in this area."

MIN-102, the lead candidate in Minoryx's pipeline, is a novel, orally bioavailable and selective PPAR gamma agonist with a superior profile for central nervous system-related diseases and good in-vivo efficacy. Phase 1 studies confirmed that MIN-102 is well tolerated and is able to cross the blood brain barrier engaging the PPAR gamma in the CNS.

"On behalf of the company, I would like to take this opportunity to thank the patients and their families, the advocacy groups, and the caregivers and their staff for achieving this fast enrollment," said Marc Martinell, CEO of Minoryx. "With the recent €21.3M Series B funding we are exploring the potential of MIN-102 in additional indications and plan to launch a new clinical study for a second orphan central nervous system indication."

More information on the trial:

https://clinicaltrials.gov/ct2/show/NCT03231878

**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 worldwide. Although it primarily affects males, heterozygous women may also develop the disease in later 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 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 in ABCD1 trigger a chain of events leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. Through its PPAR gamma activity, MIN-102 prevents such dysfunctions; it has the potential to treat both adrenomyeloneuropathy (AMN) and cerebral ALD (cALD). A phase 1 combined single- and multiple-ascending dose study was successfully completed in Q1, 2017. This confirmed that MIN-102 is well tolerated and is able to cross the blood brain barrier and engage PPAR gamma within the CNS at the same level as the one achieved in preclinical studies. MIN-102 has received Orphan Drug Designation for the treatment of X-ALD in both the EU and the US.

**About Minoryx Therapeutics**

Minoryx is a clinical stage biotech company piloting 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 is MIN-102, which may be effective in multiple CNS indications beyond X-ALD. 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 €50M.

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