Minoryx Therapeutics successfully completes phase 1 clinical trial for lead candidate MIN-102

- **MIN-102 was well-tolerated at much higher doses than those required for efficacy**
- **MIN-102 exhibited linear pharmacokinetics and no food effects were observed**
- **Blood brain barrier penetration and biomarkers confirm PPAR gamma receptor engagement in the central nervous system**
- **Phase 2/3 trial in AMN patients expected to begin in 2017**

Mataró, Barcelona Spain, March 21, 2017 – Minoryx Therapeutics, a drug development company specialized in the discovery of new drugs for orphan diseases, today announces that it has successfully completed its phase 1 trial with MIN-102.

MIN-102 targets X-linked adrenoleukodystrophy (X-ALD), a rare and chronically debilitating life threatening neurodegenerative disease. 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. 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.

The phase 1 trial was a combined single- and multiple-ascending dose study with the aim of assessing pharmacokinetics, safety and tolerability of MIN-102 in healthy male volunteers. Additionally, the trial included assessment of food effect, evaluation of brain penetration and biomarkers for PPAR Gamma engagement.

The results show that MIN-102 was generally safe and well tolerated at exposures exceeding the levels required for efficacy. No serious adverse events were observed, any adverse events were mild and similar to those observed with the placebo. Results from brain penetration and biomarker assessment confirmed earlier results, showing that MIN-102 reaches its target in the central nervous system and exerts a broad range of effects to treat various aspects of X-ALD aligned with those observed in preclinical studies. No relevant food effects were observed and pharmacokinetics showed good linearity with dose, providing a simple and convenient dosing regimen for patients.

Based on the successful completion of the phase 1 trial, a phase 2/3 trial in adult AMN patients will be launched in the coming months.

"It is very encouraging to see that MIN-102, as a PPAR gamma agonist, is able to achieve an effect size at the receptor site that cannot be achieved with the approved doses of its parent compound, pioglitazone," said Dr. Uwe Meya, CMO of Minoryx Therapeutics. "We are looking forward to initiating the phase 2/3 trial for MIN-102."

"We are very pleased with the results of the phase 1 study, which provide us with a strong scientific basis to plan and conduct clinical trials in adult patients suffering with AMN," said Dr. Marc Martinell, CEO of Minoryx Therapeutics.

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 worldwide. 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, a differentiated PPAR gamma agonist (MIN-102) that has multiple CNS indications, has successfully completed a phase 1 clinical trial and is ready to move into a phase 2/3 study with adult AMN patients. 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.
www.minoryx.com

Media Contacts & Analysts
Andrew Lloyd & Associates
Agnes Stephens – Sandra Régnavaque
agnes@ala.com / sandra@ala.com
@ALA_Group
+ 44 1273 675 100