Minoryx Therapeutics announces dosing of first US patient in phase 2/3 clinical study of MIN-102 (ADVANCE)

ADVANCE trial recruitment is progressing rapidly in Europe with over 50 percent X-ALD patients enrolled

Mataró, Barcelona, Spain, September 5, 2018- Minoryx Therapeutics, a company specializing in the development of new drugs for orphan diseases, today announces the dosing of its first US patient as part of its ongoing phase 2/3 clinical trial of MIN-102 for the treatment of adrenomyeloneuropathy (AMN). The trial enrolls adult male patients affected by AMN, the most frequent phenotype of X-linked adrenoleukodystrophy (X-ALD).

The first US patient was dosed at the Massachusetts General Hospital (MassGeneral) under the supervision of Dr. Florian Eichler. Two additional US sites will be initiating recruitment shortly: the Kennedy Krieger Institute in Baltimore, Maryland (a John Hopkins Medical Institution), with Dr. S. Ali Fatemi, and the School of Medicine of the Stanford University in California, with Dr. J.Sampson.

The first European patients were dosed at the end of 2017 and in early 2018, at the University Hospital Vall d'Hebron (Barcelona, Spain) and at the Academic Medical Center (Amsterdam, The Netherlands). Recruitment is progressing rapidly in Europe: further participating centers include the Institute of Genomic Medicine and Rare Disorders (Budapest, Hungary), Hôpital de la Pitié-Salpétrière (Paris, France), Universität Leipzig, Klinik und Poliklinik für Neurologie (Leipzig, Germany), Istituto Neurologico Carlo Besta (Milan, Italy) and the National Hospital for Neurology and Neurosurgery (London, United Kingdom).

The ADVANCE trial is a randomized, double-blinded, placebo-controlled, potentially pivotal study with an open-label extension to determine the efficacy and safety of MIN-102, a novel, orally bioavailable and selective PPAR gamma agonist with a superior profile for central nervous system-related diseases. The primary outcome is to evaluate the efficacy of MIN-102 on the progression of adrenomyeloneuropathy (AMN) in male patients, as determined by a motor function test. The trial aims to enroll more than 100 patients; results are expected at the end of 2020.

"We are proud to be the first hospital in the United States to be involved in this important clinical trial for X-ALD patients," said Dr. Eichler from MassGeneral. "We are looking forward to enrolling further patients and eagerly await results."

"We have achieved an important milestone in our trial with the first patient in the US dosed with MIN-102," said Dr. Uwe Meya, chief medical officer of Minoryx. "We are delighted that patient enrollment is already at over 50 per cent, a significant result for a clinical study in a rare disease such as X-ALD."

More information on the trial:
https://www.minoryx-advance.com/
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. The only available alternative for cALD patients is hematopoietic stem cell transplantation (HSCT), which does not prevent the development of the AMN phenotype. There is currently no pharmacological treatment available on the market for X-ALD.

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, able to cross the blood brain barrier and engage PPAR gamma within the central nervous system to the same level as in preclinical studies. MIN-102 has 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 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 is MIN-102, which has potential for multiple CNS indications beyond X-ALD. The Minoryx team consists 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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