MINORYX THERAPEUTICS FURTHER STRENGTHENS ITS SCIENTIFIC ADVISORY BOARD (SAB)

18 January, 2017

Four US and EU clinical experts in X-ALD join the board as company prepares to launch phase II/III trial in H1 2017.

Mataró, Barcelona, Spain, January 18, 2017 – Minoryx Therapeutics, a drug development company specialized in the discovery of new drugs for orphan diseases, today announces that, Dr. Patrick Aubourg, Dr. Marc Engelen, Dr. Florian Eichler and Dr. Gerald Raymond, four renowned clinical experts in X-ALD joined its scientific advisory board. The disease-specific knowledge of these clinical experts will further complement and strengthen the existing scientific advisory board consisting of Dr. David Eckland (chair), Dr. Joan Aymamí and Dr. Xavier Badia.

Minoryx Therapeutics' lead candidate, MIN-102 targets the most prevalent peroxisomal disorder, X-linked adrenoleukodystrophy (X-ALD), a rare and chronically debilitating life threatening neurodegenerative disease, currently with no available treatment. MIN-102 is a differentiated PPAR gamma agonist with a superior profile for central nervous system related diseases and excellent in-vivo efficacy. It has shown robust preclinical proof of concept in multiple animal models. Phase I studies have been initiated based on these results. A phase II/III trial in adult AMN patients has been planned. This is expected to be launched during the first half of 2017.

“We are delighted to welcome these world-leading experts in X-ALD to our board. We look forward to working with these experienced clinical investigators in developing a treatment for this devastating disease, for which there is currently no available treatment,” said Dr. Uwe Meya CMO of Minoryx Therapeutics. “Their clinical insights and experience will be very valuable to us in maximizing the potential of our lead candidate, MIN-102.”

About Dr. Patrick Aubourg

Dr. Patrick Aubourg is professor of pediatrics at the Medical University Paris-Sud (France), head of the Pediatric Neurology Department at the Hospital Bicêtre (Le Kremlin Bicêtre, France) and director of Inserm Research Unit UMR1169 at the Medical University Paris-Sud/Paris Saclay University (France). He is a member of several networks aiming to develop new therapeutic approaches in neurodegenerative diseases, in particular in the field of leukodystrophies.

About Dr. Florian Eichler

Dr. Florian Eichler is associate professor of neurology at Harvard Medical School (USA), assistant in neurology at Massachusetts General Hospital, director of the Leukodystrophy Clinic and director of the Center for Rare Neurological Diseases. Dr. Eichler studies monogenetic lipid metabolism disorders of the nervous system at the Massachusetts General Hospital and Harvard Medical School. His research focuses on the genetics of peroxisomal disorders, lipid metabolism and spatial aspects of nuclear magnetic resonance spectroscopy. Current projects include analyzing metabolic changes seen in the brain by MR measures and determining the neurotoxicity of newly discovered atypical sphingolipids.

About Dr. Marc Engelen

Marc Engelen received his MD from the University of Amsterdam (The Netherlands) in 2002. He trained as a neurologist and subsequently specialized in pediatric neurology at the Academic Medical Center (AMC) in Amsterdam. He obtained his PhD on adrenoleukodystrophy in 2012. He is currently a member of the medical staff in the Department of Neurology and the Department of Pediatrics. He has a special interest in peroxisomal disorders; the AMC was recently designated the national expert center for this group of diseases. In 2014 he received a Veni grant from NWO (The Netherlands Organisation for Scientific Research) to study the natural history of adrenoleukodystrophy and identify predictive biomarkers in the onset of cerebral ALD.

About Dr. Gerald Raymond

Dr. Raymond received his medical degree from the University of Connecticut (USA). He trained in pediatrics at Johns Hopkins University and in neurology at Massachusetts General Hospital. He completed his training in clinical genetics at Harvard University. Dr. Raymond is a pediatrician, a geneticist and a neurologist with a special interest in developmental and neurogenetic disorders affecting children and adults. He is currently working at the University of Minnesota Masonic Children's Hospital. Dr. Raymond's work focuses on the diagnosis, care and treatment of all aspects of ALD and other peroxisomal disorders. Recently, he has advanced newborn screening of ALD and clinical trials on the natural history and treatment of all aspects of ALD.

About Dr. David Eckland

David Eckland has been head of metabolic disease clinical research at GSK. His experience ranges from preclinical stages to post marketing studies. He has worked in senior positions (managing director of Takeda Europe R&D, and R&D director, Ark Therapeutics), where he developed pioglitazone, among other drugs. Dr Eckland has covered many therapeutic areas, including oncology, rheumatology, cardiovascular, urology and metabolic disease. He has supervised numerous phase IV clinical trials, interacting with commercial and business development colleagues. He has also lectured on clinical trial design at University College, London.

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, now in phase I clinical trials, is a differentiated PPAR gamma agonist (MIN-102) that has multiple CNS indications. 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

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