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Poxel Announces the Formation of its Scientific Advisory Board for Rare Metabolic Diseases

LYON, France--(BUSINESS WIRE)-- POXEL SA (Euronext – POXEL - FR0012432516), a clinical stage biopharmaceutical company developing innovative treatments for chronic diseases with metabolic pathophysiology, including non-alcoholic steatohepatitis (NASH) and rare disorders, today announced the formation of its Scientific Advisory Board (SAB) for rare metabolic diseases. The new SAB will initially focus on supporting Poxel's X-linked adrenoleukodystrophy (ALD) program.

“I am delighted to welcome this distinguished group of scientific thought leaders to Poxel’s SAB for rare metabolic diseases. These therapeutic indications, including ALD, are areas of unsurpassed unmet medical need where treatments are not available or very limited. We are grateful for their informed counsel and deep knowledge and experience that will help shape Poxel’s discovery and clinical-stage programs and further advance our mission to develop therapies for rare metabolic diseases. We look forward to continuing to collaborate with this accomplished group of advisors as we expand our clinical programs, and initiate Phase 2a studies for ALD in early 2022, with both PXL065, a novel deuterium modified thiazolidinedione and PXL770, a first-in-class direct AMPK activator,” said David E. Moller, Poxel's Chief Scientific Officer.

Poxel recently hosted its first SAB meeting on rare diseases, which led to highly productive discussions regarding the potential of its compounds and the future of its programs in ALD, including the design of its two proof-of-concept (POC) Phase 2a studies which the Company intends to initiate early 2022.

The members of the Rare Metabolic Diseases Scientific Advisory Board are the following:

Prof. Florian Eichler, MD, is an expert in inherited diseases with metabolic pathophysiology that affect the nervous system. Dr. Eichler received training in pediatric neurology and neurogenetics at Harvard and Johns Hopkins. He is currently the director of the Center for Rare Neurologic Disorders and the director of the Leukodystrophy Service at Massachusetts General Hospital (MGH), Harvard Medical School, US. His research focus is on the genetics of peroxisomal disorders, lipid metabolism, and gene therapy for neurodegenerative diseases. Dr. Eichler is also President of ALD Connect.

Prof. Marc Engelen, MD, PhD, is a clinical researcher, a specialist in pediatric neurology, gastroenterology and endocrinology, and an expert in peroxisomal disorders and leukodystrophies at Amsterdam University Medical Centers, Netherlands. Dr. Engelen

received his MD and PhD degrees at the University of Amsterdam. Along with Stephan Kemp and other members of his team, Dr. Engelen's research efforts have unveiled novel approaches to detecting, diagnosing and monitoring neurometabolic diseases including ALD.

Prof. S. Ali Fatemi, MD, is a physician-scientist leader in pediatric neurology and neurometabolic diseases. Dr. Fatemi was trained at the Medical University of Vienna, Austria and at Kennedy Krieger Institute, Johns Hopkins, US. He founded the Moser Center for Leukodystrophies and is now Chief Medical Officer at the Kennedy Krieger Institute, Baltimore, US. His research is focused on basic and translational studies pertaining to the pathophysiology of ALD.

Prof. Stephan Kemp, PhD, is a translational research expert in genetic neurometabolic diseases. He was trained at Johns Hopkins University, Kennedy Krieger Institute, and at the University of Amsterdam, where he is a longstanding faculty member. His research focuses on lipid metabolism and neurotoxicity and he has published many seminal papers on the pathobiology of ALD. He also leads efforts focused on newborn screening for rare metabolic disorders in the Netherlands.

Prof. Fanny Mochel, **MD, PhD**, is an expert in inborn errors of metabolism. She received training in genetics and neuroscience at University Pierre and Marie Curie in Paris and she is a faculty member in genetics at this university. Dr. Mochel also leads the French reference Center on Neurometabolic diseases and is co-chair of the French society for inborn of errors of metabolism and a council member of the Society for the Study of Inborn Errors of Metabolism. Her research efforts include characterization and treatment of brain energy deficiencies in neurometabolic disease, the identification of novel biomarkers, metabolomics and *in vivo* metabolic imaging, as well as therapeutic approaches targeting the Krebs cycle.

Dr. Jaspreet Singh, PhD, is a neuroscientist researcher focusing on ALD pathophysiology, neuroinflammation, biomarker development and testing novel therapeutic options. He was trained in India and at the Medical University of South Carolina, US. He is currently a faculty member in the Department of Neurology at the Henry Ford Health System in Detroit, US.

Prof. Keith Van Haren, MD, is a pediatric neurologist and an expert in ALD. He received medical and specialty training at the University of Rochester Medical School, Harvard Medical School, and Stanford University, US. Dr. Van Haren is a faculty member in Neurology and Neurological Sciences at Stanford. He cares for patients including many with ALD and also leads a laboratory focusing on the study of single-gene mutations and attendant molecular mechanisms leading to neuroinflammation in humans.

About ALD

X-linked adrenoleukodystrophy (ALD) is an orphan neurometabolic disease caused by mutations in the ABCD1 gene which encodes for a key protein that is required for metabolism of very long chain fatty acids (VLCFA) by peroxisomes (cellular organelles). ALD is the most common leukodystrophy with a prevalence similar to hemophilia – up to 1/10,000 individuals in the general population have ALD [*https://rarediseases.org*]. Forms of this disease include cerebral ALD (C-ALD) and adrenomyeloneuropathy (AMN) which is the most common form – typically occurring in adolescence through adulthood. AMN is characterized by chronic and progressive distal axonopathy involving the long tracts of the spinal cord and to a lesser extent the peripheral nerves resulting in progressive stiffness and

weakness in the legs, impaired gait and balance, incontinence, and loss of sensation. All men are affected, and many women also present with features of AMN with a later onset. C-ALD is characterized by inflammatory demyelination of cells in the brain and typically afflicts children, but many men with AMN may also develop cerebral disease; these white matter brain lesions lead to severe neurologic deficits and death. There are no approved medicines for ALD (other than glucocorticoid supplements for associated adrenal insufficiency). C-ALD when first detected in early childhood, can be treated with hematopoietic stem cell transplantation. HSCT is currently limited to early stage of C-ALD and this procedure is at risk of severe adverse reactions.

About Poxel SA

Poxel is a **clinical stage biopharmaceutical company** developing **innovative treatments for chronic serious diseases with metabolic pathophysiology**, including **non-alcoholic steatohepatitis (NASH)** and rare disorders. Poxel has clinical and earlier-stage programs from its adenosine monophosphate-activated protein kinase (AMPK) activator and deuterated TZD platforms targeting chronic and rare metabolic diseases. For the treatment of NASH, **PXL065** (deuterium-stabilized *R*-pioglitazone) is in a streamlined Phase 2 trial (DESTINY-1). **PXL770**, a first-in-class direct AMPK activator, has successfully completed a Phase 2a proof-of-concept trial for the treatment of NASH, which met its objectives. For the rare inherited metabolic disorder, adrenoleukodystrophy (ALD), the company intends to initiate Phase 2a proof of concept studies with PXL065 and PXL770 in patients with adrenomyeloneuropathy (AMN). **TWYMEEG**® (Imeglimin), Poxel's first-in-class lead product that targets mitochondrial dysfunction, has been approved and launched for the treatment of type 2 diabetes in Japan. Poxel expects to receive sales-based payments and royalties from Sumitomo Dainippon Pharma. Poxel has a strategic partnership with Sumitomo Dainippon Pharma for Imeglimin in Japan, China, South Korea, Taiwan and nine other Southeast Asian countries. The Company intends to generate further growth through strategic partnerships and pipeline development. Listed on Euronext Paris, Poxel is headquartered in Lyon, France, and has subsidiaries in Boston, MA, and Tokyo, Japan.

For more information, please visit: www.poxelpharma.com

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