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Poxel Announces PXL065 and PXL770 Granted Orphan Drug Designation from the U.S. FDA for X-Linked Adrenoleukodystrophy

LYON, France--(BUSINESS WIRE)-- POXEL SA (Euronext: POXEL - FR0012432516), a clinical stage biopharmaceutical company developing innovative treatments for serious chronic diseases with metabolic pathophysiology, including non-alcoholic steatohepatitis (NASH) and rare metabolic disorders, is pleased to announce that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to PXL065 and PXL770 for the treatment of patients with adrenomyeloneuropathy (AMN), the most common form of X-linked adrenoleukodystrophy (ALD). PXL065 is a novel, proprietary deuterium-stabilized R-stereoisomer of pioglitazone. PXL770 is a novel, first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator. Both compounds are preparing to enter into Phase 2a clinical Proof-of-Concept (POC) biomarker studies as soon as possible, subject to financing.

Poxel CEO, Thomas Kuhn, commented: "*The award of Orphan Drug Designation to both PXL065 and PXL770 by the FDA is an additional regulatory milestone for the development of these potential medicines in X-linked adrenoleukodystrophy, for which there is currently no approved therapy. This status could give either molecule a seven-year window of exclusive marketing rights following FDA approval, and, along with its recent Fast Track Designation, gives us confidence in the strategic shift into rare diseases we initiated last year. We remain excited to pursue treatments for ALD as this represents an area with very high unmet medical need. We look forward to the next steps as we are preparing for the initiation of our Phase 2a clinical studies as soon as possible. To enable this effort, we continue to pursue various financing options that will also extend our cash runway, prioritizing non-dilutive sources.*"

Orphan Drug Designation (ODD)

ODD is granted by the FDA to novel therapeutics for diseases or conditions that affect fewer than 200,000 individuals in the U.S. Orphan Drug Designation[1] gives a company a potential seven-year window of exclusive marketing rights following FDA approval, along with a reduction in certain application fees, and tax credits for expenses related to qualified clinical trials conducted after orphan designation is received.

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. Nearly all men with a diagnosis of ALD will develop AMN, 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 royalties and sales-based payments from Sumitomo Pharma. Poxel has a strategic partnership with Sumitomo 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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[1] For more information on Orphan Drug Designation, see : https://www.fda.gov/industry/developing-products-rare-diseases-conditions/designating-orphan-product-drugs-and-biological-products

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