Press Release

Poxel Announces its Participation at Patient Association Conferences in Adrenoleukodystrophy

LYON, France, June 15, 2023 – POXEL SA (Euronext: POXEL - FR0012432516), a clinical stage biopharmaceutical company developing innovative treatments for chronic serious diseases with metabolic pathophysiology, including non-alcoholic steatohepatitis (NASH) and rare metabolic disorders, today announced its participation at the ULF (United Leukodystrophy Foundation) Scientific Symposium and Family Conference on June 23rd and 24th in Itasca, Illinois, USA.

Sophie Bozec, Poxel Senior Vice President, R&D Pharmacology and Scientific Communication will present Poxel's status and plans for PXL770 and for the deuterium-modified TZD platform, using PXL065, two proprietary promising candidates, focused on the treatment of adrenoleukodystrophy (ALD), a rare and genetic disease in adults, based on robust scientific rationale and a complete preclinical package.

Poxel remains committed to discover, develop, and commercialize innovative therapies for patients suffering from serious chronic and rare diseases with underlying metabolic pathophysiology, starting with ALD. PXL770 and PXL065 have demonstrated preclinically their efficacy in adrenomyeloneuropathy (AMN) and cerebral ALD (c-ALD) in mouse and human models[1]. These two compounds are prepared to advance, subject to additional financing, into Phase 2 biomarker proof-of-concept (POC) clinical trials in male patients with AMN, the most common ALD subtype.

In line with his mission to bring therapeutic options to treat ALD, Poxel recently supported the Alex Leukodystrophy Charity during their Community Weekend, which took place from April 28th to May 1st in Birmingham, England. This event brings together leukodystrophy sufferers and their families, alongside doctors, researchers and scientists from around the world to discuss leukodystrophies.

[1] Preclinical study results were published in prestigious journals:

- In *The Journal of Inherited Metabolic Disease ("JIMD")*: "Therapeutic potential of deuterium-stabilized (R)-pioglitazone - PXL065 - for X-linked adrenoleukodystrophy".
- In *The Journal of Pharmacology and Experimental Therapeutics ("JPET")*: "Beneficial effects of the direct AMP-Kinase activator PXL770 in in vitro and in vivo models of X-Linked Adrenoleukodystrophy".

POXEL LISTED EURONEXT

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. For the treatment of NASH, **PXL065** (deuterium-stabilized *R*-pioglitazone) met its primary endpoint in a streamlined Phase 2 trial (DESTINY-1). In rare diseases, development of **PXL770**, a first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator, is focused on the treatment of adrenoleukodystrophy (ALD) and autosomal dominant polycystic kidney disease (ADPKD). **TWYMEEG®** (Imeglimin), Poxel's first-in-class product that targets mitochondrial dysfunction, is marketed for the treatment of type 2 diabetes in Japan by Sumitomo Pharma and Poxel expects to receive royalties and sales-based payments. Poxel has a strategic partnership with Sumitomo Pharma for Imeglimin in Japan, China, and eleven other Asian countries. 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

All statements other than statements of historical fact included in this press release about future events are subject to (i) change without notice and (ii) factors beyond the Company's control. These statements may include, without limitation, any statements preceded by, followed by or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can

have," "likely," "should," "would," "could" and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results or performance to be materially different from the expected results or performance expressed or implied by such forward-looking statements. The Company does not endorse or is not otherwise responsible for the content of external hyperlinks referred to in this press release.

Contacts - Investor relations / Media

Aurélie Bozza
Investor Relations & Communication Senior Director
aurelie.bozza@poxelpharma.com
+33 6 99 81 08 36

Elizabeth Woo
Senior Vice President, Investor Relations & Communication
elizabeth.woo@poxelpharma.com

NewCap
Emmanuel Huynh or Arthur Rouillé
poxel@newcap.eu
+33 1 44 71 94 94