September 27, 2021

Poxel Announces its Participation at Upcoming Scientific Conferences Related to Adrenoleukodystrophy (ALD)

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 that the Poxel team will participate at several upcoming scientific conferences related to X-linked adrenoleukodystrophy (ALD), a severe orphan neurometabolic disease with no approved therapies.

Poxel's scientific team will present data and plans pertaining to ALD that align with the recently announced new strategic direction of increasing Poxel's focus on rare metabolic diseases.

Phase 2a clinical Proof of Concept (POC) biomarker studies, examining PXL065 and PXL770 in patients with adrenomyeloneuropathy (AMN), the most common subtype of ALD which affects the nervous system and adrenal glands, are planned to initiate in early 2022 with data readouts anticipated by year end 2022.

Poxel is committed to focus its pipeline on high value, rare metabolic indications and NASH, with the goal of creating pipeline synergies, maximizing resources, and driving shareholder value.

Upcoming Scientific Conferences

- **11th International Meeting on AMPK – Evian-les-Bains, France** (in person)
  Date: September 26-30, 2021

  Poxel will deliver the following oral presentations:

  - on Monday, September 27, 3:00pm CET, *"Characterization of a first-in-class direct AMPK activator, PXL770, for NASH and other metabolic disorders: From Preclinical to Clinical"* by Sophie Bozec, PhD, Senior Vice President, R&D Pharmacology and Scientific Communication
  - on Tuesday, September 28, 9:30am CET, *"Potential therapeutic utility of direct AMPK activators for X-linked adrenoleukodystrophy"* by Pierre-Axel Monternier, Senior Manager, Pharmacology

- **World Congress of Neurology (WCN)** (virtual)

Date: October 3-7, 2021

Poxel will deliver a poster presentation entitled: *"Validation of Direct AMP Kinase Activation for Treatment of X-linked Adrenoleukodystrophy"*

- **National Organization for Rare Disorders (NORD) Summit** (virtual)
  Date: October 18-19, 2021

  Poxel will deliver poster presentations entitled: *"(R)-pioglitazone – PXL065 – for Treatment of X-Linked Adrenoleukodystrophy (ALD)"* and *"Validation of Direct AMP Kinase (AMPK) Activation for Treatment of X-Linked Adrenoleukodystrophy (ALD)"*

- **ALD Connect Annual Meeting** (virtual)
  Date: November 12-13, 2021

  Members of Poxel's scientific team will participate and present at this conference.

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 thiazolidinedione (D-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, X-linked 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

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.

View source version on businesswire.com:
https://www.businesswire.com/news/home/20210926005039/en/

Poxel SA
Catherine David
Investor Relations & Communication Manager
catherine.david@poxelpharma.com
+33 7 64 57 61 78

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

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

Investor relations / Media - EU/US
Trophic Communications
Stephanie May or Valeria Fisher
poxel@trophic.eu
+49 171 185 56 82 or +49 175 804 1816

Investor relations / Media - France
NewCap
Emmanuel Huynh or Arthur Rouillé
poxel@newcap.eu
+33 1 44 71 94 94

Source: Poxel SA