



## **Poxel Presents Results of Two Clinical Studies on its Direct AMP Kinase Activator, the PXL770, at the International Liver Congress (ILC)<sup>™</sup> 2021**

- **Pr. Kenneth Cusi presented the results of the STAMP-NAFLD 12-week, randomized, controlled Phase 2a trial of PXL770 in 120 presumed NASH patients – selected as a “Best of ILC” abstract**
- **Pr. Vlad Ratziu presented the results from a 4-week PK/PD target engagement study of PXL770**

LYON, France--(BUSINESS WIRE)-- [POXEL SA](#) (Euronext – POXEL - FR0012432516), a biopharmaceutical company focused on the development of innovative treatments for metabolic disorders, including type 2 diabetes and non-alcoholic steatohepatitis (NASH) presented the results of two studies at the European Association for the Study of the Liver (EASL) International Liver Congress<sup>™</sup>, held from June 23-26, 2021.

- On June 25, during an oral presentation [Abstract #427] in the “NAFLD: therapy” session, Pr. Kenneth Cusi (U. of Florida) presented the results of the 12-week, randomized, controlled Phase 2a trial in 120 presumed NASH patients, with or without T2DM, which evaluated three dosing regimens of PXL770, Poxel’s lead direct AMP kinase activator, versus placebo. The results showed that treatment with PXL770 at 500 mg QD resulted in significant reductions in mean liver fat content and alanine transaminase (ALT) levels (vs. baseline). Greater effects were observed in patients with coexisting Type 2 diabetes (T2D, 41-47% of each group): -27% reduction in liver fat content at 500 mg QD vs. baseline; an increase in the proportion of responders (>30% reduction in liver fat); dose-responsive and significant mean decreases in ALT and aspartate transaminase (AST) levels vs. placebo. In the T2D patients, significant placebo-adjusted decreases were observed in fasting plasma glucose and HbA1c (-0.64%) despite well-controlled baseline fasting levels (121-144 mg/dL and 6.6-7.1%, respectively), along with improvements in commonly used fasting indices of insulin sensitivity (HOMA-IR and QUICKI scores). PXL770 was well tolerated with an acceptable safety profile.

- On June 23, Pr. Vlad Ratziu (Université Pierre et Marie Curie and the Hôpital Pitié-Salpêtrière Medical School, Paris) presented a poster [Abstract #159] with the results from a 4-week study designed to assess the PK profile, safety, and target engagement of PXL770 (500 mg QD) in 12 patients (plus 4 on placebo) with elevated liver fat and insulin resistance. The observed PK profile and safety results were consistent with previous results obtained in

Phase I studies with healthy subjects. PXL770 treatment produced a significant suppression of *de novo* lipogenesis, indicating target engagement, along with a significant improvement in glycemia (total and incremental glucose AUC) following an oral glucose challenge test (OGTT). Improvements in several indices of insulin sensitivity were also observed.

“Taken together, the results of these two clinical studies provide strong evidence of efficacy for PXL770 in patients with non-alcoholic fatty liver disease, insulin resistance, and Type 2 diabetes,” commented Pascale Fouqueray, MD, PhD, Executive Vice President, Clinical Development and Regulatory Affairs at Poxel. “The results are also notable as they represent the first reported clinical data for any direct AMP kinase activator in humans. Given a favorable safety profile, along with evidence of metabolic benefits with this lead molecule, we are also excited by the prospect of pursuing this mechanism in other important clinical indications in addition to NASH – potentially including adrenoleukodystrophy.”

## **About Poxel SA**

Poxel is a dynamic biopharmaceutical company that uses its extensive expertise in developing innovative drugs for metabolic diseases, with a focus on type 2 diabetes and non-alcoholic steatohepatitis (NASH), and selected rare inherited disorders including adrenoleukodystrophy. In its mid-to-late-stage pipeline, the Company is currently advancing three drug candidates; several earlier-stage opportunities are also underway. Imeglimin, Poxel’s first-in-class lead product, targets mitochondrial dysfunction. Poxel has a strategic partnership with Sumitomo Dainippon Pharma for TWYMEEG® (Imeglimin) in Japan, China, South Korea, Taiwan and nine other Southeast Asian countries. A new drug application for TWYMEEG for the treatment of type 2 diabetes, was approved in Japan. After successfully completing a Phase 2a proof-of-concept trial for the treatment of NASH, which met its primary endpoint and study objectives, for PXL770, a first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator, Poxel plans to initiate a Phase 2b program in the second half of 2021. PXL770 could also have the potential to treat additional metabolic diseases. PXL065 (deuterium-stabilized R-pioglitazone), is in a streamlined Phase 2 trial for the treatment of NASH. Poxel also has additional earlier-stage programs from its AMPK activator and deuterated thiazolidinediones (TZD) platforms targeting chronic and rare metabolic diseases. 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](http://www.poxelpharma.com)

In the context of the COVID-19 outbreak, which was declared a pandemic by the World Health Organization (WHO) on March 12, 2020, the Company is regularly reviewing the impact of the outbreak on its business.

As of the date of this press release, and based on publicly available information, the Company has not identified the occurrence of any material negative effect on its business due to the COVID-19 pandemic that remains unresolved. However, the Company anticipates that the COVID-19 pandemic could have further material negative impact on its business operations. The worldwide impact of COVID-19 may notably affect the Company’s internal organization and efficiency, particularly in countries where it operates and where confinement measures are implemented by the authorities. In addition, COVID-19 may

impact market conditions and the Company's ability to seek additional funding or enter into partnerships. Particularly, delays in the supply of drug substance or drug products, in the initiation or the timing of results of preclinical and/or clinical trials, as well as delays linked to the responsiveness of regulatory authorities could occur, which could potentially have an impact on the Company's development programs and partnered programs. The Company will continue to actively monitor the situation.

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.

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