May 17, 2022

Poxel Provides Corporate Update and Reports Cash and Revenue for the First Quarter 2022

- **Fast Track and Orphan Drug Designation for PXL065 and PXL770 in adrenoleukodystrophy (ALD) granted by the Food and Drug Administration (FDA)**
- **Phase 2 study results for PXL065 (DESTINY-1) in NASH expected in Q3 2022**
- **As of March 31, 2022, cash and cash equivalents were EUR 24.0 million (USD 26.7 million)**

LYON, France--(BUSINESS WIRE)-- 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 provided a corporate update and announced its cash position and revenue for the first quarter ended March 31, 2022.

"*Receiving FDA Orphan Drug Designation for PXL065 and PXL770 in ALD, along with the recent Fast Track Designation for both compounds, gives us confidence that we can expedite clinical development for ALD which represents an area with very high unmet medical need. We are preparing for the launch of our two Phase 2a proof-of-concept clinical trials which we plan to initiate as soon as possible. We look forward to sharing topline results from our Phase 2 DESTINY-1 study in NASH for PXL065 in the third quarter of 2022," said Thomas Kuhn, Chief Executive Officer of Poxel. "We continue to pursue various financing options to extend our cash runway, prioritizing non-dilutive sources. We remain focused on developing our clinical candidates and demonstrating their value in the field of metabolic diseases*."

Commercial Update

TWYMEEG® (Imeglimin)

- As of March 31, 2022, royalty revenue to Poxel based on TWYMEEG net sales in Japan under the Sumitomo Pharma license agreement has been limited following TWYMEEG's recent commercial launch on September 16, 2021. TWYMEEG's initial commercial uptake has been affected by restrictions in Japan on prescribing any new drug in its first year of commercialization, and conditions related to COVID-19, which have reduced the frequency of physician visits and limited the extensive prescriber education efforts required for any launch of an innovative drug with a new mechanism of action. However, as a result of Sumitomo Pharma's promotional activities and efforts

since launch, TWYMEEG is very well known among prescribers.

Clinical Updates

NASH

- PXL065 (deuterium-stabilised R-pioglitazone) is in a Phase 2 study (DESTINY-1). Results from this 36-week, randomized, double-blind, placebo-controlled, parallel group, dose-ranging study designed to assess efficacy and safety are anticipated in Q3 2022. The goal of DESTINY-1 is to identify the optimal dose or doses of PXL065 to advance into a Phase 3 registration trial for the treatment of noncirrhotic biopsy-proven NASH patients.

Rare metabolic diseases

- In ALD, Phase 2a biomarker proof-of-concept (POC) clinical trials of PXL065 and PXL770 are expected to initiate as soon as possible, subject to additional financing. Two identical studies will enroll adult male patients with adrenomyeloneuropathy (AMN), the most common ALD subtype. The POC studies will evaluate the pharmacokinetics, safety and efficacy of PXL065 and PXL770 after 12 weeks of treatment based on relevant disease biomarkers, such as the effect on very long chain fatty acids (VLCFA), the characteristic plasma marker of the disease.
- In February and April, the FDA awarded Fast Track Designation (FTD) to PXL065 and PXL770 respectively, for ALD. The FDA grants FTD to investigational drugs which treat a serious or life-threatening condition, and which fill an unmet medical need. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially better than available therapy. The key benefits of FTD comprise enhanced access to the FDA, with regular and more frequent opportunities for consultation and discussion.

Significant Events after the Period

- During the 2nd quarter, the FDA granted Orphan Drug Designation (ODD) to PXL065 and PXL770 for ALD. ODD confers 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.

First Quarter 2022 Financial Update

As of March 31, 2022, cash and cash equivalents were EUR 24.0 million (USD 26.7 million), as compared to EUR 32.3 million (USD 36.6 million) as of December 31, 2021. Net financial debt (excluding IFRS16 impacts and derivative debts) was EUR 10.1 million as of March 31, 2022, as compared to EUR 2.6 million as of December 31, 2021.

EUR (in thousands)	**Q1 2022**	**Q4 2021**
Cash	24,043	28,753
Cash equivalents	-	3, 534

Total cash and cash equivalents*	**24,043**	**32,287**

Unaudited data

** Net financial debt (excluding IFRS 16 impacts and derivative debts) was EUR 10,1 million at the end of Q1 2022 as compared to EUR 2.6 million at the end of Q4 2021*

Based on:

i. the cash position at March 31, 2022,
ii. the current development plan of the Company including 1) the completion of its ongoing Phase 2 NASH trial for PXL065 (DESTINY-1) but excluding 2) the two identical Phase 2a clinical proof-of-concept (POC) biomarker studies for PXL065 and PXL770 in adrenomyeloneuropathy (AMN),
iii. the cash forecast for the year 2022 approved by the Board of Directors of the Company, that 1) does not include, as a conservative approach, any net sales from Imeglimin in Japan and 2) includes an amount of EUR 4,813 thousands for the repayments of the loan with IPF partners until December 2022, and
iv. a strict control of its operating expenses,

the Company expects that its resources will be sufficient to fund its operations and capital expenditure requirements through at least December 31, 2022. However, the Company is subject to certain financial covenants[1] related to its debt with IPF Partners which could be potentially breached in Q3 2022.

The Company is actively pursuing various financing options, which include dilutive and non-dilutive sources, as well as discussions with IPF partners.

First Quarter 2022 Revenue

Poxel reported EUR 32 thousand revenue for the quarter ended March 31, 2022, as compared to no revenue during the corresponding period in 2021.

Revenue for the first quarter of 2022 reflects JPY 4.4 million (EUR 32 thousand) of royalty revenue from Sumitomo Pharma which represents 8% of TWYMEEG net sales in Japan. Based on the current forecast, Poxel expects to receive 8% royalties on TWYMEEG net sales in Japan through the Sumitomo Pharma fiscal year 2022 (April 2022 to March 2023). As part of the Merck Serono licensing agreement, Poxel will pay Merck Serono a fixed 8% royalty based on the net sales of Imeglimin, independent of the level of sales.

EUR (in thousands)	**Q1 2022 3 months**	**Q1 2021 3 months**
Sumitomo Pharma Agreement	32	-
Other	-	-
Total revenues	**32**	**-**

Unaudited data

Planned Presentations and Participation at the Following Upcoming Events

- European Renal Association, May 25-28
- 5th Global NASH Congress, May 26-27
- Jefferies Global Healthcare Conference, June 8-10
- JMP Securities 2022 Life Sciences Conference, June 15-16

Next Financial Press Release : Second Quarter 2022 Financial Update on August 30, 2022.

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 from Sumitomo Pharma royalties and sales-based payments. 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] Under these financial covenants the Company should maintain minimum cash position of the higher of i) ten million euros and ii) the sum of the consolidated debt service of the Company plus the amount of cash required to be spent by the Company as part of its operations, in each case for the following 6-month period.

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