October 20, 2021

Poxel Provides Corporate Update and Reports Cash and Revenue for the Third Quarter and Nine Months 2021

- **TWYMEEG® (Imeglimin hydrochloride) launched in Japan on September 16 for the treatment of type 2 diabetes**
- **Milestone payment for TWYMEEG's approval in Japan of JPY 1.75 billion (approximately EUR 13.2 million, USD 15.8 million)[1] paid by Sumitomo Dainippon Pharma to Poxel in July 2021**
- **Patient enrollment in PXL065 Phase 2 (DESTINY-1) trial in NASH completed in September with results expected in Q3 2022**
- **Phase 2a clinical Proof of Concept (POC) biomarker studies of PXL065 and PXL770 in X-linked adrenoleukodystrophy (ALD) planned to initiate in early 2022 with data expected by year end 2022**
- **As of September 30, 2021, cash and cash equivalents were EUR 37.2 million (USD 43.2 million)**

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 provided a corporate update and announced its cash position and revenue for the third quarter and the nine months ended September 30, 2021.

"During the third quarter, we continued to make significant progress and accomplished a number of important clinical and corporate objectives. The TWYMEEG® approval last June and subsequent launch on September 16 have increased our financial position and will continue to bolster our financial foundation with associated future royalties and sales-based payments. We are extremely proud to have brought TWYMEEG® to patients in Japan through our fruitful partnership with Sumitomo Dainippon Pharma. In line with our commitment towards NASH, in September, we completed patient enrollment in the PXL065 Phase 2 trial where we anticipate reporting topline results in Q3 2022," said Thomas Kuhn, CEO of Poxel.

"For the remainder of this year, we are fully committed to advancing our programs, particularly to support the important clinical milestones of initiating the Phase 2a clinical Proof of Concept biomarker studies of PXL065 and PXL770 in ALD in early 2022 and completing the PXL065 Phase 2 trial in NASH in Q3 2022. Our recent accomplishments highlight our capability to deliver on our goals which are aligned with Poxel's mission to develop innovative therapeutics for patients living with metabolic diseases," Mr. Kuhn added.

Clinical Updates

Rare metabolic diseases (PXL065 and PXL770)

- Poxel is planning to initiate two phase 2a clinical Proof of Concept (POC) biomarker studies of PXL065 and PXL770 in X-linked adrenoleukodystrophy (ALD) in early 2022 and all regulatory processes are on track. Data are expected by year end 2022.
- In September, Poxel attended the 11th International Meeting on AMPK in Evian-les-Bains, France, and Pierre-Axel Monternier, Senior Manager, Pharmacology, delivered a presentation: *"Potential therapeutic utility of direct AMPK activators for X-linked adrenoleukodystrophy."*
- In early October, at the World Congress of Neurology (WCN), Poxel delivered a poster presentation entitled *"Validation of Direct AMP Kinase Activation for Treatment of X-linked Adrenoleukodystrophy."*
- In mid-October, at the National Organization for Rare Disorders (NORD) Summit, Poxel delivered two 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)."*

NASH (PXL065 and PXL770)

- In September, the PXL065 Phase 2 trial (DESTINY-1) in NASH completed patient enrollment with 123 noncirrhotic biopsy-proven NASH patients across multiple clinical sites in the US in a 36- week, randomized, dose-ranging, double-blind, placebo-controlled, parallel group study designed to assess the efficacy and safety of PXL065. Results from this Phase 2 study are anticipated in Q3 2022. The goal of this trial 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.
- In September, at the 11th International Meeting on AMPK in Evian-les-Bains, France, Poxel Senior Vice President, R&D Pharmacology and Scientific Communication, Sophie Bozec, PhD, presented: *"Characterization of a first-in-class direct AMPK activator, PXL770, for NASH and other metabolic disorders: from preclinical to clinical."*

Type 2 Diabetes (TWYMEEG®/Imeglimin)

- On September 16, TWYMEEG was launched in Japan for the treatment of type 2 diabetes by Sumitomo Dainippon Pharma. TWYMEEG is Poxel's first product to reach commercialization and Japan is the first country where the product has been approved. Poxel received a milestone payment of JPY 1.75 billion (EUR 13.2 million, USD 15.8 million)[1] from Sumitomo Dainippon Pharma in July for the approval of TWYMEEG. Additionally, as part of the license agreement with Sumitomo Dainippon Pharma, Poxel is entitled to receive escalating royalties on net sales and sales-based payments in accordance with sales goals.

Corporate Updates

- In September, Sylvie Bertrand joined the Poxel management team as Vice President, Human Resources, based in Lyon, France. Sylvie has an extensive experience in HR, having worked in different companies where she led efforts to define and implement

HR resources, strategy and processes.

Third Quarter and Nine Months Ended September 30, 2021 Cash and Revenue

Cash

As of September 30, 2021, cash and cash equivalents were EUR 37.2 million (USD 43.2 million), as compared to EUR 40.2 million (USD 49.4 million) at December 31, 2020. Cash and cash equivalents net of financial liabilities (excluding IFRS16 impacts and derivative debts) were EUR 1.7 million as of September 30, 2021, as compared to EUR 17.1 million at December 31, 2020. In July 2021, Poxel received a EUR 13.2 million[2] milestone payment from its partner Sumitomo Dainippon Pharma for the Imeglimin approval obtained on June 23, 2021.

EUR (in thousands)	**Q3 2021**	**Q4 2020**
Cash	32,058	15,588
Cash equivalents	5,173	24,615
Total cash and cash equivalents*	**37,231**	**40,203**

Unaudited data

**Cash and cash equivalents net of financial liabilities were EUR 1.7 million at the end of Q3 2021 and EUR 17.1 million at the end of Q4 2020.*

Nine Months 2021 Revenue

Poxel reported revenues of EUR 13.3 million for the nine months ended September 30, 2021, as compared to EUR 6.6 million during the corresponding period in 2020.

Revenue for the first nine months of 2021 mostly reflects the JPY 1.75 billion (EUR 13.2 million)[3] milestone payment paid by Sumitomo Dainippon Pharma to Poxel in July 2021. No revenue was reported in Q3.

Revenue for the first nine months of 2020 included the milestone payment that Poxel received from Sumitomo Dainippon Pharma for the submission of the Imeglimin J-NDA. In a lesser extent, it also included an allocated portion of the EUR 36.0 million upfront payment received from Sumitomo Dainippon Pharma relating to the strategic corporate partnership announced on October 30, 2017, as well as the residual Imeglimin Phase 3 program costs in Japan incurred during the first nine months of 2020 that were re-invoiced to Sumitomo Dainippon Pharma.

EUR *(in thousands)*	**H1 2021 6 months**	**Q3 2021 3 months**	**Sept. 2021 9 months**	**H1 2020 6 months**	**Q3 2020 3 months**	**Sept. 2020 9 months**
Roivant Agreement	-	-	-	13	5	18
Sumitomo Dainippon Pharma Agreement	13,274	-	13,274	6,359	195	6,554
Other		-				
Total revenues	**13,274**	**-**	**13,274**	**6,372**	**199**	**6,571**

Unaudited data

Planned Presentations and Participation at the Following Upcoming Events

- ALD Connect Annual Meeting (virtual) - November 12-13, 2021
- AASLD The Liver Meeting (virtual) - November 12-15, 2021
- Jefferies London Healthcare Conference (in-person and virtual) – November 16-19, 2021
- 5th Annual NASH Summit (virtual) – November 29-December 2, 2021
- ODDO BHF Forum (virtual) – January 6-11, 2022
- H.C. Wainwright BioConnect 2022 (virtual) – January 10-13, 2022

Next Financial Press Release: Fourth Quarter 2021 Financial Statement expected on February 16, 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 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.

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[1] Currency exchange rate at the date of the approval.
[2] Currency exchange rate at the date of the approval.
[3] Currency exchange rate at the date of the approval.

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