September 23, 2021

Poxel Reports Financial Results for First Half 2021 and Provides a Corporate Update

- **Strengthened financial position with the approval of TWYMEEG® (Imeglimin hydrochloride) for the treatment of type 2 diabetes in Japan:**
  - **Allowed Poxel to draw the third and final tranche of the IPF loan for EUR 13.5 million - included in the Q2 cash and cash equivalents of EUR 36.9 million**
  - **Triggered a milestone payment of JPY 1.75 billion (approximately EUR 13.2 million, USD 15.8 million)[1] from Sumitomo Dainippon Pharma to Poxel which was paid in July 2021 (not included in Q2 cash and cash equivalents)**

- **Increasing strategic focus on rare metabolic diseases with continued commitment to non-alcoholic steatohepatitis (NASH):**
  - **Patient enrollment in PXL065 DESTINY Phase 2 trial in NASH was completed in September 2021 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**

- **Webcast on Thursday, September 23, at 12:00 pm ET / 6:00 pm CEST (in French), 1:00 pm ET / 7:00 pm CEST (in English)**

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 its financial results for the period ended June 30, 2021 and provided a corporate update.

“Thus far, 2021 has proven to be a pivotal year for Poxel as exemplified by the approval and subsequent product launch of TWYMEEG® in Japan. This first product approval highlights our proven capabilities to deliver upon our corporate and clinical goals and to bring improved metabolic therapeutics to patients. This achievement has strengthened our near-term financial position and will continue to enhance our financial foundation with associated future royalties and sales-based payments,” said Thomas Kuhn, CEO of Poxel. “We also look forward to harnessing the full potential of our strategic shift with an increased focus on rare metabolic diseases, in addition to our ongoing commitment to NASH. Poxel is well-positioned to leverage its existing platforms to develop therapeutics in rare metabolic diseases,” Mr. Kuhn concluded.

Corporate Highlights

- The June 23 approval of TWYMEEG® in Japan, Poxel's first product to reach commercialization, triggered two financing events:

  1. In June 2021, Poxel received the third and final tranche of the IPF loan for EUR 13.5 million which is included in the second quarter cash and cash equivalents of EUR 36.9 million.
  2. In July 2021, Poxel received a milestone payment of JPY 1.75 billion (approximately EUR 13.2 million, USD 15.8 million)[1] from Sumitomo Dainippon Pharma.

- Poxel appointed Dr. John Kozarich to the Board of Directors during the June 23, 2021, Annual and Extraordinary General Meeting, where he was also appointed as Chair of the Scientific Committee of the Board. Dr. Kozarich has over 40 years of experience in the biopharmaceutical industry and academia. Bpifrance Participations transitioned off as Board observer, effective July 9, 2021.
- In June, Poxel conducted the Annual and Extraordinary General Meeting. The shareholders approved all resolutions that were recommended by the Board of Directors. For further information, please visit: https://www.poxelpharma.com/en_us/investors/shareholder-information/annual-general-meeting-documents.

Clinical Development Updates

Type 2 Diabetes (TWYMEEG®/Imeglimin)

- On June 23, the new drug application for the 500mg TWYMEEG® tablets for the treatment of type 2 diabetes, was approved in Japan. The first-in-class drug with a unique dual mechanism of action was approved as a monotherapy and/or as an additional treatment to other glucose-lowering therapy regimens. Approval was based on positive results from various preclinical and clinical studies, including the Phase 3 TIMES (Trials of IMeglimin for Efficacy and Safety) program managed jointly by Poxel and Sumitomo Dainippon Pharma. The program included three pivotal trials to evaluate TWYMEEG®'s efficacy and safety in over 1,100 patients. TWYMEEG® met its primary endpoints and objectives, exhibiting a favorable safety and tolerability profile.
- Three presentations on Imeglimin were included in an oral session for large clinical studies at the 64th Annual Meeting of the Japan Diabetes Society held in May. The presentations focused on the analysis and interpretation of clinical data derived from Phase 2 and Phase 3 (TIMES program) trials with Imeglimin in Japanese patients with type 2 diabetes.
- In January, Poxel regained all Imeglimin rights from Metavant. In the US and Europe, Poxel continues to explore options to move Imeglimin forward in Phase 3 in patients with type 2 diabetes with moderate to severe chronic kidney disease (CKD), including partnering activities.

Rare metabolic diseases (PXL065 and PXL770)

- In X-linked adrenoleukodystrophy (ALD), a rare disease affecting the nervous system and adrenal glands, Phase 2a clinical POC biomarker studies examining PXL065 and

PXL770 are planned to initiate in early 2022 with data readouts anticipated by year end 2022. Two identical studies will enroll adult male ALD patients with adrenomyeloneuropathy (AMN), the most common form of the disease, and observe the effect of PXL065 and PXL770 over 12 weeks of treatment on pharmacokinetics, safety, and efficacy using relevant biomarkers, including the impact on elevated very long chain fatty acids (VLCFA), the hallmark plasma marker of the disease.
- In April, Dr. Sophie Bozec, Senior Vice President, R&D Pharmacology and Scientific Communication at Poxel, delivered a presentation at the Mitochondria-Targeted Drug Development Summit highlighting Poxel's scientific focus and deep expertise in mechanisms modulating cellular energy homeostasis and addressing mitochondrial dysfunction – including showcasing the potential of Poxel's AMP-kinase (AMPK) activator and deuterium-stabilized thiazolidinedione (dTZD) platforms for rare disorders where mitochondrial dysfunction is a major pathogenic component.

NASH (PXL065 and PXL770)

- PXL065, deuterium-stabilized R-pioglitazone, is being evaluated for safety and efficacy in a streamlined Phase 2 trial, DESTINY1, for the treatment of NASH. Patient enrollment was completed in September 2021, with topline data anticipated approximately one year later. This 36-week trial in noncirrhotic biopsy-proven NASH patients will assess three doses of PXL065 compared to placebo in 123 patients. The results will be used to help identify the dose or doses for a Phase 3 registrational trial.
- Future development of PXL770, a first-in-class, oral direct AMPK activator, in NASH will be assessed pending results from the PXL065 Phase 2 trial in NASH and both Phase 2a POC studies in ALD.
- Dr. Kenneth Cusi and Dr. Vlad Ratziu each delivered presentations on PXL770 clinical studies at the European Association for the Study of the Liver (EASL) International Liver Congress™ (ILC), held from June 23-26, 2021. Dr. 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. Dr. Ratziu presented the results from a 4-week PK/PD target engagement study of PXL770 in NAFLD patients.

Subsequent Events after the Period

- In July, Poxel announced it will pursue a strategic direction to focus its pipeline on high value, rare metabolic indications, to complement its commitment to NASH, with the goal of creating pipeline synergies, maximizing resources, and driving shareholder value.
- In July, ENYO Pharma SA announced positive results for Vonafexor (EYP001), an FXR agonist that was licensed from Poxel in 2015, in a Phase 2a study in NASH patients over 12 weeks and topline interim results from two ongoing Phase 2a studies in chronic hepatitis B patients.
- In August, Elizabeth Woo was appointed as Senior Vice President, Investor Relations, Public Relations & Corporate Communications. Elizabeth brings over 25 years of investor relations and biotech communications experience in initial public offerings, follow-on financings, market launches, and commercialized products. She is based in Boston.
- On September 16, Poxel's strategic partner, Sumitomo Dainippon Pharma, launched

TWYMEEG® 500mg tablets for the treatment of type 2 diabetes in Japan. Japan is the first country where the product has been approved. As part of the license agreement with Sumitomo Dainippon Pharma, Poxel is entitled to receive escalating double-digit royalties on net sales and sales-based payments (based on Poxel's current forecast) of up to JPY 26.5 billion (approximately EUR 200 million, USD 230 million)[2] in accordance with sales goals.

- In September, data for PXL770 was published online by Hepatology Communications, titled "Direct AMPK Activation Corrects NASH in Rodents Through Metabolic Effects and Direct Action on Inflammation and Fibrogenesis."

First Half 2021 Financial Results (IFRS standards)

Revenue

Poxel reported revenues of EUR 13.3 million for the six months ended June 30, 2021, as compared to EUR 6.4 million during the corresponding period in 2020.

Revenue for the first half of 2021 mostly reflects the JPY 1.75 billion (EUR 13.2 million) milestone payment from Sumitomo Dainippon Pharma which Poxel received in July 2021.

EUR (in thousands)	**H1 2021 6 months**	**H1 2020 6 months**
Roivant Agreement		13
Sumitomo Dainippon Pharma Agreement	13,274	6,359
Total revenues	**13,274**	**6,372**

Limited review procedures have been performed by the auditors and the issuance of the report is in process.

Income Statement

Poxel devotes the bulk of its resources to research and development (R&D) activities. R&D expenses totaled EUR 14.7 million for the first half of 2021, as compared to EUR 12.6 million for the corresponding period in 2020. R&D expenses for the first half of 2021 primarily reflect the clinical study costs incurred for the phase 2 DESTINY study evaluating PXL065 in NASH. To a lesser extent, they also reflect the Phase 2a clinical program cost of PXL770 for the treatment of NASH and the regulatory costs incurred over the period for TWYMEEG®, for which the Company obtained marketing approval in June 2021.

R&D expenses are net of the R&D Tax Credit (CIR) that resulted in an income of EUR 1.6 million for the first half of 2021 as compared to the same level for the corresponding period of 2020.

General and administrative expenses totaled EUR 5.4 million for the first half of 2021, as compared to EUR 6.0 million for the first half of 2020.

The financial loss amounted to EUR 1.2 million for the first half of 2021, as compared to an income of EUR 0.2 million for the first half of 2020. It primarily reflected the interests attached to the Company indebtedness.

The net result for the financial period ending June 30, 2021, was a net loss of EUR 8.0 million, as compared to a net loss of EUR 12.1 million in the corresponding period in 2020.

Condensed Income Statement

EUR (in thousands)	**HY 2021 6 months**	**HY 2020 6 months**
Revenue	13,274	6,372
Research and development expenses*	(14,683)	(12,580)
General and administrative expenses	(5,443)	(5,983)
Operating gain (loss)	(6,851)	(12,191)
Financial income (loss)	(1,178)	249
Income tax	-	(118)
Net income (loss)	**(8,029)**	**(12,060)**

**Net of R&D tax credit*

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Cash

As of June 30, 2021, cash and cash equivalents were EUR 36.9 million (USD 43.9 million), as compared to EUR 40.2 million (USD 49.4 million) on December 31, 2020. Cash and cash equivalents were fully offset by financial liabilities (excluding IFRS16 impacts and derivative debts) as of June 30, 2021. Cash and cash equivalents net of financial liabilities (excluding IFRS16 impacts and derivative debts) were EUR 17.1 million on December 31, 2020. In July 2021, Poxel received a EUR 13.2[3] million milestone payment from its partner Sumitomo Dainippon Pharma for the Imeglimin approval, which is not reflected in the June 30, 2021 cash update.

EUR (in thousands)	**Q2 2021**	**Q4 2020**
Cash	20,431	15,587
Cash equivalents	16,490	24,616
Total cash and cash equivalents*	**36,921**	**40,203**

**Cash and cash equivalents were fully offset by financial liabilities (excluding IFRS16 impacts and derivative debts) as of June 30, 2021. Cash and cash equivalents net of financial liabilities (excluding IFRS16 impacts and derivative debts) were EUR 17.1 million on December 31, 2020.*

Limited review procedures have been performed by the auditors and the issuance of the report is in process.

The webcasts will be held on September 23, 2021:

- In French, at 12:00 pm ET (New York) / 6:00 pm CEST (Paris time).

To register for the webcast:
https://us02web.zoom.us/webinar/register/WN_o4kretFCS8uz7iHv0iLmxg

- In English, at 1:00 pm ET (New York) / 7:00 pm CEST (Paris time).
  To register for the webcast:
  https://us02web.zoom.us/webinar/register/WN_Flka34pnTdGsTj_fJSvGIw

A slide presentation to accompany the webcast will be available in the Investors section of Poxel website.

Participants will be able to submit questions through the webcast chat during the event.

Following the live call, a replay will be available for 90 days on https://www.poxelpharma.com/en_us/investors/company-information/corporate-presentations

Planned Presentations and Participation at the Following Upcoming Events

- International AMPK meeting, Evian, France, September 26-30, 2021
- HealthTech Innovation Days, France, October 4-5, 2021
- World Congress of Neurology, October 3-7, 2021
- H.C. Wainwright NASH Conference, October 12, 2021
- National Organization for Rare Diseases (NORD) Rare Disease Summit, October 18-19, 2021

Next Financial Press Release: Third Quarter 2021 Financial and Corporate Update on October 21, 2021

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 (DESTINY1). 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 agreement (30 Oct 2017).

[3] Currency exchange rate at the date of the approval

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