July 21, 2021

Poxel Provides Corporate Update and Reports Cash and Revenue for the Second Quarter and First Half 2021

- **As of June 30, 2021, cash and cash equivalents were EUR 36.9 million (USD 43.9 million) which includes EUR 13.5 million from the third and final tranche of IPF loan triggered by the recent approval of the Company's lead drug candidate TWYMEEG® (Imeglimin) in Japan**
- **Poxel to receive JPY 1.75 billion (approximately EUR 13.2 million, USD 15.8 million)[1] milestone payment from Sumitomo Dainippon Pharma in Q3 2021 following TWYMEEG approval; product launch in Japan expected in 2021**
- **In rare metabolic diseases, PXL065 and PXL770 Phase 2a clinical Proof-of-Concept (POC) studies in X-linked adrenoleukodystrophy (ALD)expected to initiate in early 2022 with data expected in Q4 2022**
- **In non-alcoholic steatohepatitis (NASH), patient enrollment in PXL065 DESTINY Phase 2 trial expected to be complete in Q3 2021 with results expected in Q3 2022**

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 disorders, today announced its cash position and revenue for the second quarter and first half of 2021.

"Following our recent achievements, we are excited to leverage our proven capabilities and existing platforms to execute upon our new strategic direction - an increased focus on rare metabolic diseases, along with our continued commitment to NASH," said Thomas Kuhn, CEO of Poxel. "The recent TWYMEEG approval in Japan is a major milestone for Poxel. It enhances our financial foundation allowing us to pursue our development goals in rare metabolic indications where we can be more efficient with our resources and more expediently deliver novel medicines to patients with an even stronger potential to create significant value for the benefit of our shareholders. This approval is the culmination of years of clinical development work and a strong validation for both our R&D capabilities and the international footprint that we have established."

Clinical Development Updates

Rare metabolic diseases:

- In ALD, Phase 2a clinical POC biomarker studies of PXL065 and PXL770 are planned to initiate in early 2022, with data expected by year end 2022. Two identical studies will enroll adult male ALD patients with the most common form of the disease (adrenomyeloneuropathy – AMN) and assess 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 disease.

NASH:

- In NASH, PXL065, deuterium-stabilized *R*-pioglitazone, is in a streamlined Phase 2 trial (DESTINY1). Patient screening is finished and enrollment is now expected to be completed in Q3 2021, with topline data anticipated approximately one year later. This Phase 2, 36-week trial in noncirrhotic biopsy-proven NASH patients will assess three doses of PXL065 compared to placebo in at least 120 patients. The results will be used to help identify the dose or doses for a Phase 3 registration trial.
- Initiation of the NASH Phase 2b trial for PXL770, a first-in-class, oral direct AMPK activator, will be postponed, pending results from the ongoing PXL065 Phase 2 trials in NASH and both Phase 2a biomarker studies in AMN.
- Results of two studies for PXL770 were presented at the European Association for the Study of the Liver (EASL) International Liver Congress™, held from June 23-26, 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. PXL770 was observed to produce improvements in liver fat content and liver enzymes with a greater response in patients with co-existing type 2 diabetes (T2D); in these patients, additional improvements in glycemia were observed. PXL770 was observed to be safe and well tolerated.
- Pr. Vlad Ratziu presented the results from a 4-week PK/PD target engagement study of PXL770. Observed PK profile and safety results were consistent with previous results from 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.

TWYMEEG (Imeglimin)

- On June 23, the new drug application for TWYMEEG tablets 500mg[2] (International Nonproprietary Name (INN): Imeglimin hydrochloride), for the treatment of type 2 diabetes, was approved in Japan, the first country in the world to approve it. The TWYMEEG approval is supported by numerous 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, which included three pivotal trials to evaluate the efficacy and safety of TWYMEEG in over 1,100 patients. In all three trials, TWYMEEG met its primary endpoints and objectives and was observed to exhibit a favorable safety and tolerability profile. TWYMEEG is a first-in-class drug with a unique dual mechanism of action for the treatment of type 2 diabetes across the continuum of the current treatment paradigm, both as a monotherapy or as an add-on to other glucose lowering therapies.

- At the 64th Annual Meeting of the Japan Diabetes Society in May, three presentations on Imeglimin were included in an oral session for large clinical studies. These presentations focused on the analysis and interpretation of clinical data derived from Phase 2 and Phase 3 (TIMES program) trials with Imeglimin where Japanese patients with type 2 diabetes were enrolled and studied.
- 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.

Corporate Update

- The June 23rd approval of Company's lead drug candidate, Imeglimin (TWYMEEG) in Japan triggered two financing events:

  1. In June, 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 Q3, Poxel will receive a JPY 1.75 billion (approximately EUR 13.2 million, USD 15.8 million)[3] milestone payment from Sumitomo Dainippon Pharma.

- On June 23, 2021, Poxel held its annual general meeting. The shareholders approved all the 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.
- Dr. John Kozarich was appointed to the Board of Directors during the June 23, 2021 general assembly meeting, and also became the chair of the scientific committee of the Board. Bpifrance Participations transitioned off as a Board observer, effective July 9, 2021.

Second Quarter and First Half 2021 Cash and Revenue

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) at 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.

EUR (in millions)	**Q2 2021**	**Q4 2020**
Cash	20.4	15.6
Cash equivalents	16.5	24.6
Total cash and cash equivalents*	**36.9**	**40.2**

Unaudited data
**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.*

Second Quarter and First Half 2021 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 will receive in the third quarter.

Revenue in the first half of 2020 included a JPY 500 million (EUR 4.1 million) milestone payment that Poxel received from Sumitomo Dainippon Pharma upon submission of the Imeglimin J-NDA in July 2020. To 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 half of 2020 that were re-invoiced to Sumitomo Dainippon Pharma.

EUR (in millions)	Q1 2021 3 months	Q2 2021 3 months	**H1 2021 6 months**	Q1 2020 3 months	Q2 2020 3 months	**H1 2020 6 months**
Sumitomo Agreement	-			1.5	4.8	6.4
Other	-	13.3	13.3	-	-	-
Total revenues	-	13.3	**13.3**	1.5	**4.8**	**6.4**

Unaudited data

Planned Presentations and Participation at the Following Upcoming Virtual Events

- H.C. Wainwright 23rd Annual Global Investment Conference - September 13-15, 2021
- International AMPK meeting, Evian, France – September 26-30, 2021

Next Financial Press Release: First Half financial results on September 23, 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 (DESTINY). 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. In 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 for the treatment of type 2 diabetes in Japan. With this approval, Poxel is entitled to receive milestones, 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.

---

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

[2] Dosage and administration: In general, for adults, 1,000 mg of Imeglimin hydrochloride is orally administered twice daily in the morning and evening.

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

View source version on businesswire.com:
https://www.businesswire.com/news/home/20210721005760/en/

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

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