October 20, 2020

Poxel Provides Corporate Update and Reports Cash and Revenue for the Third Quarter and Nine Months 2020

- **Imeglimin New Drug Application in Japan (J-NDA) was submitted for the treatment of type 2 diabetes in July 2020 and a target launch is expected in fiscal year 2021[1]; a milestone payment of EUR 4 million was received in Q3 2020 from Sumitomo Dainippon Pharma**
- **Imeglimin Phase 3 TIMES results were presented at the 56th European Association for the Study of Diabetes (EASD) meeting; Phase 2b, Phase 3 TIMES results, and additional safety data were presented at the 63rd Annual Meeting of Japanese Diabetes Society (JDS)**
- **PXL770 Phase 2a trial for the treatment of NASH met its primary efficacy endpoint and trial objectives, and it was observed to be safe and well tolerated**
- **PXL770 profile supports development in NASH and also further evaluation for combination use, as well as utility of adenosine monophosphate-activated protein kinase (AMPK) activation in other chronic and rare metabolic diseases**
- **PXL065 Phase 2 trial was initiated in biopsy-proven NASH patients in September 2020; streamlined development with a single Phase 2 trial given knowledge of pioglitazone, including data in NASH, and 505(b)(2) regulatory pathway, which offers the opportunity for an efficient and lower risk development program**
- **As of September 30, 2020, cash and cash equivalents were EUR 41.5 million (USD 48.6 million)**

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), today provided a corporate update and announced its cash position and revenue for the third quarter and the nine months ended September 30, 2020.

“During the third quarter, we continued to make significant progress and accomplished a number of important clinical and corporate objectives, including reporting positive results from a Phase 2a proof-of-concept trial for PXL770, demonstrating its potential in NASH. These results are the first human clinical assessment of a direct AMPK activator and support longer-term evaluation of important histological endpoints, such as inflammation and fibrosis, and exploring subpopulations for further differentiation. The data also demonstrate that AMPK activation may lead to broader utility for the treatment of other chronic and rare metabolic diseases. In addition, we initiated a streamlined Phase 2 trial for PXL065 in NASH

and strengthened our cash position with non-dilutive funding from a milestone payment of EUR 4 million for the Imeglimin New Drug Application (NDA) submission in Japan and the recent PGE loan of EUR 6 million from the French government," said Thomas Kuhn, CEO of Poxel.

"For the remainder of this year, we expect several upcoming milestones and events including finalization of the PXL770 Phase 2b clinical trial design, presentations for PXL770 and PXL065 at several scientific meetings as well as publishing results in peer-reviewed scientific journals and additional preclinical data related to our AMPK and deuterated-TZD platforms. Furthermore, our partner, Metavant, is in discussions with the U.S. Food and Drug Administration (FDA) regarding the Imeglimin Phase 3 program in type 2 diabetes patients with chronic kidney disease (CKD) stages 3b/4," added Thomas Kuhn, CEO of Poxel.

Clinical Development Updates

Imeglimin (Type 2 Diabetes)

- The Company worked closely with Sumitomo Dainippon Pharma on activities related to the J-NDA for Imeglimin for the treatment of type 2 diabetes, which was submitted in late July to the Pharmaceuticals and Medical Devices Agency (PMDA) to request approval for manufacturing and marketing. A target launch is expected in 2021[2]. The J-NDA approval would trigger a milestone payment of EUR 14.2 million ($16.6 million)[3].
- Phase 3 TIMES 2 and TIMES 3 trial results were presented at the 56th EASD meeting, demonstrating that Imeglimin met its primary endpoints and objectives and was observed to exhibit a favorable safety and tolerability profile.
- Imeglimin results focused on safety benefits were presented at the 63rd JDS meeting. Speakers included Professor Kohjiro Ueki, MD, PhD, Director, Diabetes Research Center, the National Center for Global Health and Medicine, Tokyo, Japan and Professor Wataru Ogawa, MD, PhD, Professor, Division of Diabetes and Endocrinology, Department of Internal Medicine, Kobe University, Graduate School of Medicine, Kobe, Japan. In the Phase 2b and Phase 3 TIMES trials, Imeglimin was observed to have a favorable safety profile at the dose of 1,000 mg with similar frequency and types of adverse events as in the placebo group, and specifically appeared to be unlikely to cause hypoglycemia. In addition, new preclinical results showed that Imeglimin has a novel mechanism of action regulating mitochondrial bioenergetics, with a partial inhibition of complex I and no inhibition on mitochondrial glycerol 3-phosphate dehydrogenase (mGPDH), a driver of lactate accumulation, which further differentiates Imeglimin from metformin.
- Metavant, the Company's partner for the U.S. and Europe, is in discussions with the FDA regarding the Imeglimin Phase 3 program in type 2 diabetes patients with chronic kidney disease (CKD) stages 3b/4.

PXL770 (NASH)

- The Phase 2a trial for the treatment of NASH met its primary efficacy endpoint; PXL770-treated patients achieved statistically significant improvement in the relative decrease in liver fat mass measured by magnetic resonance imaging-estimated proton density fat fraction (MRI-PDFF) at 12-weeks with a greater response in patients with

type 2 diabetes[4].

- In the Phase 2a trial, key secondary measures in PXL770-treated patients included statistically significant observed improvements in liver enzymes alanine transaminase (ALT) and hemoglobin A1c (HbA1c).
- In the Phase 2a trial, PXL770 was observed to be safe and well tolerated. The results support the development in NASH and also further evaluation for combination use with other agents. Additional data are currently being analyzed and the Company is working with key opinion leaders to finalize the Phase 2b clinical trial design for PXL770.
- Phase 2a results also support utility of AMPK activation in other chronic and rare metabolic diseases.
- Preclinical results presented at the 56th EASD meeting showed PXL770 was observed to improve renal and cardiac disease in a preclinical model demonstrating utility for NASH co-morbidities and additional indications driven by metabolic dysfunction.
- Poxel anticipates presenting new data for PXL770 in a peer-reviewed format at scientific meetings and in scientific journals during the fourth quarter of 2020.

PXL065 (NASH)

- In early September 2020, a streamlined Phase 2 study was initiated to evaluate PXL065 in at least 120 biopsy-proven NASH patients with the aim to identify the optimal dose or doses for a Phase 3 registration trial. The results from this study are anticipated to be available during the first half of 2022.
- Poxel anticipates presenting new data for PXL065 in a peer-reviewed format at scientific meetings and in scientific journals during the fourth quarter of 2020.

Additional Development Opportunities

- The Company is currently evaluating additional research and development opportunities from its AMPK activation and deuterated TZD platforms as well as external opportunities with a focus on chronic and rare metabolic diseases.

Corporate Update

- In October, Poxel received financing approval from BNP Paribas, Bpifrance and CIC Lyonnaise de Banque for a total of EUR 6 million in the form of state-guaranteed loans (Prets Garantis par l'Etat, or PGE in France) in the context of the COVID-19 pandemic.

Third Quarter and Nine Months Ended September 30, 2020 Cash and Revenue

Cash

As of September 30, 2020, cash and cash equivalents were EUR 41.5 million (USD 48.6 million), as compared to EUR 37.2 million (USD 41.8 million) at December 31, 2019. Cash and cash equivalents net of financial liabilities (excluding IFRS16 impacts and derivative debts) were EUR 24.5 million as of September 30, 2020, as compared to EUR 27.4 million at December 31, 2019. In October 2020, Poxel received a EUR 6.0 million PGE loan from the French government, which is not reflected in the September 30, 2020 cash update.

EUR (in thousands)	**Q3 2020***	**Q4 2019**

Cash	19,738	18,161
Cash equivalents	21,794	19,026
Total cash and cash equivalents**	**41,532**	**37,187**

**Unaudited data*
***Cash and cash equivalents net of financial liabilities (excluding IFRS 16 impacts and derivative debts) were EUR 24.5 million at the end of Q3 2020 and EUR 27.4 million at the end of Q4 2019.*

Nine Months 2020 Revenue

Poxel reported revenues of EUR 6.6 million for the nine months ended September 30, 2020, as compared to EUR 26.0 million during the corresponding period in 2019 (historical).

Revenue for the first nine months of 2020 includes 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 and the milestone payment that Poxel received from Sumitomo Dainippon Pharma for the submission of the Imeglimin J-NDA. Both the allocated portion of the upfront payment and the re-invoiced costs of the Phase 3 **T**rials of **IM**eglimin for **E**fficacy and **S**afety (TIMES) program are recognized based on the accounting percentage of the completion of this program, which has been completed, and therefore led to the decrease in revenue.

EUR (in thousands)	H1 2020 6 months	Q3 2020 3 months*	**Sept 2020 9 months***	H1 2019 6 months		Q3 2019 3 months		**Sept 2019 9 months**	
				Adjusted**	Historical	Adjusted**	Historical	**Adjusted****	**Histo**
Roivant Agreement	13	5	18	155	155	52	52	**207**	**20**
Sumitomo Agreement	6,359	195	6,554	18,909	22,914	2,971	2,771	**21,879**	**25,**
Other	-	-	-	100	100	-	-	**100**	**10**
Total revenues	**6,372**	**199**	**6,571**	**19,164**	**23,169**	**3,023**	**2,823**	**22,186**	**25,**

**Unaudited data*
***Proforma, as if the Company applied the standalone selling price method in FY19.*

Note: A change in the accounting policy of revenue recognition method was reported as part of the fiscal year 2019 financial statements in a press release dated February 12, 2020. This resulted in an adjustment to the Sumitomo Dainippon Pharma partnership revenue recognition for the previous years. For more information, please visit: https://www.poxelpharma.com/en_us/investors/news-events/press-releases/detail/144/poxel-reports-financial-update-for-cash-and-revenue-for-the

This change in accounting policy had no impact on Poxel's cash flows.

Planned Presentations and Participation at the Following Upcoming Events

- B. Riley Liver Disease Therapeutics Day, October 29, 2020 (virtual)
- Direct Dirigeants Event, November 3, 2020 (in-person conference)
- American Association for the Study of Liver Diseases (AASLD), The Liver Meeting®, November 13-17, 2020 (virtual)
- ALD Connect Annual Meeting, November 13-15, 2020 (virtual)
- Bryan Garnier Healthcare Conference, November 16, 2020 (virtual)
- Jefferies Virtual London Healthcare Conference, November 17-19, 2020 (virtual)
- Oddo Digital Tech40 Forum, November 24-25, 2020 (virtual)
- NASH Summit Boston, December 15-17, 2020 (virtual)

Next Financial Press Release: Fourth Quarter 2020 Financial Statement expected on February 11, 2021

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)**. In its mid-to-late stage pipeline, the Company is currently advancing three drug candidates as well as earlier-stage opportunities. **Imeglimin**, Poxel's first-in-class lead product, targets mitochondrial dysfunction. Poxel has a strategic partnership with Sumitomo Dainippon Pharma for Imeglimin in Japan, China, South Korea, Taiwan and nine other Southeast Asian countries. A Japanese new drug application (J-NDA) is under review by the Pharmaceuticals and Medical Devices Agency (PMDA) to request approval for the manufacturing and marketing of Imeglimin for the treatment of type 2 diabetes. Poxel also established a partnership with Roivant Sciences for Imeglimin's development and commercialization in countries outside of the partnership with Sumitomo Dainippon Pharma, including the U.S. and Europe. **PXL770**, a first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator, has successfully completed a Phase 2a proof-of-concept trial for the treatment of NASH. The Phase 2a trial met its primary endpoint and study objectives. PXL770 could also have the potential to treat additional metabolic diseases. **PXL065** (deuterium-stabilized R-pioglitazone), an MPC inhibitor, is in a single Phase 2 trial for the treatment of NASH. Poxel also has additional earlier-stage programs from its AMPK activator and deuterated 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.

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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[1] Year noted is Fiscal Year from April 2021 to March 2022, which is Sumitomo Dainippon Pharma's Fiscal Year.

[2] Year noted is Fiscal Year from April 2021 to March 2022, which is Sumitomo Dainippon Pharma's Fiscal Year.

[3] Converted at the exchange rates as of June 30, 2020.

[4] Prevalence of type 2 diabetes in patients with NASH estimated to be 47% (Younossi ZM et al, *Hepatology 64*, 73–84, 2016).

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Source: Poxel SA