October 7, 2019

Poxel Reports Financial Results for the Third Quarter and Nine Months 2019 and Provides Corporate Update

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 announced its cash position and revenue for the third quarter and nine months ended September 30, 2019.

As of September 30, 2019, cash and cash equivalents were EUR 36.8 million (USD 40.1 million), as compared to EUR 66.7 million (USD 76.4 million) as of December 31, 2018. Cash and cash equivalents net of financial liabilities were EUR 30.5 million as of September 30, 2019, as compared to EUR 52.5 million as of December 31, 2018.

The cash balance ending September 30, 2019, does not include the 2018 R&D tax credit payment of EUR 3.6 million, which is expected to be received by year-end. Poxel's 2019 cash-burn has been consistent as planned and the cash balance at year-end is expected to be above EUR 30.0 million.

EUR (in millions)	Q3 2019	Q4 2018
Cash	8.8	7.3
Cash equivalents	28.0	59.4
Total cash and cash equivalents*	**36.8**	**66.7**

Unaudited data
** Cash and cash equivalents net of financial liabilities were EUR 52.5 million at the end of Q4 2018 and EUR 30.5 million at the end of Q3 2019*

Poxel reported revenues of EUR 26.0 million for the nine months ended September 30, 2019, as compared to revenues of EUR 55.0 million during the same period in 2018.

EUR (in millions)								
	Q1	Q2	Q3	**9 months ended Sept. 30**	Q1	Q2	Q3	**9 months ended Sept. 30**

	2019	2019	2019	**2019**	2018	2018	2018	**2018**
Roivant Agreement	-	0.2	-	0.2	8.1	-		8.1
Sumitomo Agreement	14.9	8.0	2.8	25.7	10.2	19.2	17.5	46.9
Other	-	0.1	-	0.1				
Total revenues	**14.9**	**8.3**	**2.8**	**26.0**	**18.3**	**19.2**	**17.5**	**55.0**

Unaudited data

Revenue for the nine months ended September 30, 2019 mostly reflects a 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 Imeglimin Phase 3 program costs in Japan incurred during the first nine months of 2019 that were re-invoiced to Sumitomo Dainippon Pharma. Both the upfront payment from Sumitomo Dainippon Pharma and the re-invoiced costs of the Phase 3, **T**rials of **IM**eglimin for **E**fficacy and **S**afety (TIMES) program are recognized according to the percentage of completion for this program.

“Following several significant achievements in the first half of 2019, including positive top-line results from the Imeglimin Phase 3 TIMES 1 and TIMES 3 trials in Japan, I am pleased to report that during the third quarter of 2019 we continued to advance our two NASH programs and initiated clinical trials for PXL770 and PXL065. Both of these trials are expected to generate results in the fourth quarter of 2019 that will help to determine future development strategies for the programs. In addition, positive top-line results from the Metavant trial for Imeglimin in patients with type 2 diabetes and chronic kidney disease were also announced,” said Thomas Kuhn, CEO of Poxel.

“The fourth quarter of 2019 will be very important with several significant milestones anticipated for our three clinical development programs. For Imeglimin, we expect to complete the Phase 3 TIMES program in Japan and report the results from the TIMES 2 and TIMES 3 36-week open label portion of the trial. For PXL770, we anticipate results from the pharmacokinetic and pharmacodynamic trial, including effects on target pathways. For PXL065, we will be meeting with the U.S. Food and Drug Administration to discuss the next steps for development in NASH, including the registration program. In addition, we will be presenting PXL065 data at the American Association for the Study of Liver Diseases meeting and expect to report results from the Phase 1b trial, which should help to support dose selection for the registration program,” added Thomas Kuhn.

Clinical Development Update

Imeglimin Update

- In July 2019, positive top-line results were reported from the Metavant trial in patients with type 2 diabetes and chronic kidney disease (CKD) stages 3b/4. Imeglimin was observed to demonstrate a favorable safety and tolerability profile and the pharmacokinetics (PK) and pharmacodynamics (PD) data were consistent with previous Poxel data.
- Metavant plans to work with regulatory authorities and aims to initiate a Phase 3 program in patients with type 2 diabetes and CKD stages 3b/4 in the U.S. and Europe.
- In September 2019, detailed Phase 3 results from the TIMES 1 program, which evaluated the efficacy, safety and tolerability of Imeglimin in Japanese patients with

type 2 diabetes as well as data supporting the safety and efficacy of Imeglimin in various patient populations, were featured in a symposium session at the 55th Annual Meeting of the European Association for the Study of Diabetes.
- Phase 3 results from the TIMES 2 and TIMES 3 36-week open-label portion of the trial are expected around the end of 2019.

PXL770 Update

- In July 2019, a trial evaluating the PK and PD of PXL770 was initiated and results are expected in the fourth quarter of 2019. This study will assess the full PK profile and PD effect on target pathways and metabolic parameters and is being conducted in parallel with the ongoing Phase 2a efficacy and safety study, which is expected to report results in the second quarter of 2020.

PXL065 Update

- In September 2019, a Phase 1b multiple ascending dose trial was initiated and results are expected in the fourth quarter of 2019. The Phase 1b multiple ascending dose trial is designed to evaluate safety, tolerability and PK and support dose selection for the registration program.
- Poxel will meet with the U.S. Food and Drug Administration (FDA) early in the fourth quarter 2019 to discuss the registration program and 505(b)(2) regulatory pathway, which has the potential for expedited development and accelerated regulatory approval.
- At the upcoming American Association for the Study of Liver Diseases (AASLD) held on November 8-12, 2019 in Boston, Massachusetts, Poxel will present a poster presentation for PXL065.

Planned Presentations and Participation at the Following Upcoming Events

- Paris Large & Midcap Event, October 14-15, 2019, Paris, France
- HC Wainwright 3rd Annual NASH Investor Conference, October 21, 2019, New York, NY
- Licensing Executives Society (LES) 2019 Annual Meeting, October 20-23, 2019, Phoenix, AZ
- BioNetwork Partnering Summit, October 23-25, 2019, Laguna Niguel, CA
- Gilbert Dupont NASH Conference, October 29, 2019, Paris, France
- American Association for the Study of Liver Diseases (AASLD) poster presentation for PXL065, November 8-12, 2019, Boston, MA
- William Blair Boston Innovation Day, November 12, 2019, Boston, MA
- Bio-Europe, November 11-13, 2019, Hamburg, Germany
- China Healthcare Summit 2019, The Bridge to Innovation, Shanghai, China, November 18-20, 2019
- Jefferies Global Healthcare Conference, November 20-21, 2019, London, UK
- World Congress on Insulin Resistance, Diabetes and Cardiovascular Disease, December 4-7, 2019, Los Angeles, CA

Next Financial Press Release: Fourth Quarter Financial Update, February 12, 2020

About the Phase 3 TIMES Program

TIMES (**T**rials of **Im**eglimin for **E**fficacy and **S**afety), the Phase 3 program for Imeglimin for the treatment of type 2 diabetes in Japan, consists of three pivotal trials involving over 1,100 patients. The TIMES program is a joint development effort between Poxel and Sumitomo Dainippon Pharma Co., Ltd. The companies entered into a strategic partnership in October 2017 for the development and commercialization of Imeglimin in Japan, China, South Korea, Taiwan and nine other Southeast and East Asian countries[1]. The TIMES program includes the following three trials that will be performed using the dose of 1,000 mg twice daily:

TIMES 1: A Phase 3, 24-week, double-blind, placebo-controlled, randomized, monotherapy trial to assess the efficacy, safety and tolerability of Imeglimin in Japanese patients with type 2 diabetes, using the change in HbA1c as the primary endpoint. Secondary endpoints of the trial include fasting plasma glucose, other standard glycemic and non-glycemic parameters. The TIMES 1 trial met its primary and secondary endpoints and the top-line data was reported on April 9, 2019.

TIMES 2: A Phase 3, 52-week, open-label, parallel-group trial to assess the long-term safety and efficacy of Imeglimin in Japanese patients with type 2 diabetes. In this trial, Imeglimin will be administrated orally as a monotherapy or combination therapy with existing hypoglycemic agents, including a DPP4 inhibitor, SGLT2 inhibitor, biguanide, sulphonylurea, glinide, alpha-glucosidase inhibitor, thiazolidine and GLP1 receptor agonist. The TIMES 2 results are expected around the end of 2019.

TIMES 3: A Phase 3, 16-week, double-blind, placebo-controlled, randomized trial with a 36-week open-label extension period to evaluate the efficacy and safety of Imeglimin in combination with insulin in Japanese patients with type 2 diabetes and inadequate glycemic control on insulin therapy. The TIMES 3 16-week portion of the trial met its primary endpoint with a favorable safety and tolerability profile observed and the top-line data was reported on June 25, 2019. The TIMES 3 36-week open label results are expected around the end of 2019.

About Imeglimin:
Imeglimin is the first clinical candidate in a new chemical class of oral agents called Glimins by the World Health Organization. Imeglimin has a unique mechanism of action (MOA) that targets mitochondrial bioenergetics. Imeglimin acts on all three key organs which play an important role in the treatment of type 2 diabetes: the pancreas, muscles, and the liver, and it has demonstrated glucose lowering benefits by increasing insulin secretion in response to glucose, improving insulin sensitivity and suppressing gluconeogenesis. This MOA has the potential to prevent endothelial and diastolic dysfunction, which can provide protective effects on micro- and macro-vascular defects induced by diabetes. It also has the potential for protective effect on beta-cell survival and function. This unique MOA offers the potential opportunity for Imeglimin to be a candidate for the treatment of type 2 diabetes in almost all stages of the current anti-diabetic treatment paradigm, including monotherapy or as an add-on to other glucose lowering therapies.

About PXL770
PXL770 is a first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator. AMPK is a central regulator of multiple metabolic pathways leading to the control of lipid metabolism, glucose homeostasis and inflammation. Based on its central metabolic role, targeting AMPK offers the opportunity to pursue a wide range of indications to treat

chronic metabolic diseases, including diseases that affect the liver, such as non-alcoholic steatohepatitis (NASH)[2].

About PXL065
PXL065, formerly DRX-065, is deuterium-stabilized R-pioglitazone. Pioglitazone is the most extensively studied drug for NASH and has demonstrated "resolution of NASH without worsening of fibrosis" in a Phase 4 trial[3]. Pioglitazone is the only drug recommended for biopsy-proven NASH patients by the Practice Guidelines published by the American Association for the Study of Liver Diseases (AASLD) and the European Association for the Study of the Liver (EASL)[4]. Pioglitazone's use for NASH, however, has been limited due to the PPARγ-related side effects, which include weight gain, bone fractures and fluid retention.

Pioglitazone is a 1:1 mixture of two mirror-image compounds (R- and S-stereoisomers) that interconvert *in vivo*. Using deuterium, we stabilized each stereoisomer and characterized their dramatically different pharmacological properties. In *in vitro* studies, PXL065 has been shown to target MPC as an inhibitor. In preclinical models, PXL065 exhibits the anti-inflammatory activity and NASH efficacy associated with pioglitazone with little or no weight gain or fluid retention, side effects which are associated with the S-stereoisomer. Based upon preclinical and Phase 1 results to date, PXL065 is expected to exhibit a better therapeutic profile than pioglitazone for NASH.

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. Together, with its partner Sumitomo Dainippon Pharma, Poxel is conducting the Phase 3 **T**rials of **IM**eglimin for **E**fficacy and **S**afety (TIMES) program for the treatment of type 2 diabetes in Japan. 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, is in a Phase 2a proof-of-concept program for the treatment of NASH. PXL770 could also have the potential to treat additional metabolic diseases. **PXL065** (deuterium-stabilized R-pioglitazone), a mitochondrial pyruvate carrier (MPC) inhibitor, is in Phase 1 clinical testing and being developed for the treatment of NASH. Poxel also has additional earlier-stage programs targeting metabolic, specialty and rare 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.

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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] Including Indonesia, Vietnam, Thailand, Malaysia, The Philippines, Singapore, Republic of the Union of Myanmar, Kingdom of Cambodia and Lao People's Democratic Republic.
[2] Source: Smith B. K et al., (2016) Am J Physiol Endocrinol Metab 311, E730 – E740.
[3] Cusi, et al., Ann Intern Med. 2016, 165(5), 305-315).
[4] J Hepatol. 2016, 64(6),1388-402; Hepatology 2018, 67, 328-357.

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