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Poxel Announces First Quarter 2019 Financial 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 first quarter ended March 31, 2019.

As of March 31, 2019, cash and cash equivalents were EUR 59.0 million (USD 66.3 million), as compared with EUR 66.7 million (USD 76.4) as of December 31,2018.

EUR millions	Q1 2019	Q4 2018
Cash	3.7	7.3
Cash equivalents	55.3	59.4
Total cash and cash equivalents*	**59.0**	**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 47.4 millions at the end of Q1 2019*

Poxel reported revenues of EUR 14.9 million for the quarter ended March 31, 2019, as compared with revenues of EUR 18.3 million during the same period in 2018.

EUR millions	Q1 2019	Q1 2018
Roivant Agreement		8.1
Sumitomo Agreement	14.9	10.2
Total revenues	**14.9**	**18.3**

Unaudited data

The revenue reflects a portion of the EUR 36 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 quarter that were re-invoiced to Sumitomo Dainippon Pharma. Both the upfront

payment from Sumitomo Dainippon Pharma and 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.

“This year will be very important for Poxel. There will be several significant milestones that have the potential for substantial value creation. For Imeglimin, key milestones include the Phase 3 data results beginning early second quarter with the announcement of the TIMES 1 results. We expect that these results will be followed by the TIMES 3 results mid-year for the 16-week, double-blind, placebo-controlled part of the study. Further Phase 3 data from the TIMES 2 and TIMES 3 studies are expected to be reported during the fourth quarter of 2019,” said Thomas Kuhn, CEO of Poxel. “We have been working very closely with our partner Sumitomo Dainippon Pharma in preparing for the Japanese New Drug Application for Imeglimin for the treatment of type 2 diabetes, which is a milestone targeted for 2020 with product launch anticipated in 2021.”

“For Imeglimin in the US, the study in patients with type 2 diabetes and chronic kidney disease stages 3b/4 is progressing well. Following successful completion of this study, Metavant plans to meet with the US Food and Drug Administration with the goal of advancing into a Phase 3 program in this specific patient population,” continued Thomas Kuhn.

“For our two clinical-stage programs for the treatment of NASH, we recently announced initiation of a Phase 2a program for PXL770. The Phase 2a study will include efficacy and safety assessment in patients who likely have NASH. During the second quarter of 2019, we expect to initiate a separate pharmacokinetic and pharmacodynamic study for PXL770. Data results from these studies are anticipated to be announced beginning later this year,” continued Thomas Kuhn.

“For PXL065, during the second quarter of 2019, we plan to initiate the Phase 1b multiple ascending dose study, and results are expected during the third quarter of 2019. Following completion of the Phase 1 program, we plan to initiate a pivotal Phase 2 program in biopsy-proven NASH patients in the fourth quarter or early 2020.”

Planned Presentations and Participation at the Following Upcoming Events

The International Liver Congress, European Association for the Study of the Liver, April 10-19, 2019, Vienna, Austria

BIO International Convention 2019, June 3-6, 2019, Philadelphia, PA

Jefferies 2019 Global Healthcare Conference, June 4-7, 2019, New York City, NY

American Diabetes Association Annual Meeting, June 7-11, 2019, San Francisco, CA

JMP Securities Life Sciences Conference, June 19-20, 2019, New York City, NY

Next Financial Press Release: Second Quarter 2019 Financial Statement expected on July 15, 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 liver, muscles and the pancreas, 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 the 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 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 study 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 will include other standard glycemic and non-glycemic parameters.

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

TIMES 3: A Phase 3, 16-week, double-blind, placebo-controlled, randomized study 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.

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)[1].

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[2]. 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).[3] 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 uses its development expertise in metabolism to advance a pipeline of drug candidates focused on the treatment of metabolic disorders, including type 2 diabetes and non-alcoholic steatohepatitis (NASH). We have successfully completed the Phase 2 clinical program for our first-in-class lead product, Imeglimin, which targets mitochondrial dysfunction, in the U.S., Europe and Japan. Together, with our partner Sumitomo Dainippon Pharma, we are 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. Our partner Roivant Sciences is responsible for Imeglimin's development and commercialization in countries outside of Poxel's 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 and being developed for the treatment of NASH. Poxel also has additional earlier-stage programs, including deuterated drug candidates for metabolic, specialty and rare diseases. We intend to generate further growth through strategic partnerships and pipeline development. (Euronext: POXEL, www.poxelpharma.com)

1. Source: Smith B. K et al., (2016) Am J Physiol Endocrinol Metab 311, E730 – E740

2. Cusi, *et al.*, Ann Intern Med. 2016, 165(5), 305-315)

3. J Hepatol. 2016, 64(6),1388-402; Hepatology 2018, 67, 328-357

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