October 16, 2018

Poxel Announces Third Quarter and Nine Months 2018 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 third quarter and nine months ended September 30, 2018.

As of September 30, 2018, cash and cash equivalents were EUR 76.8 million (USD 88.8 million). The cash position reported reflects the DeuteRx upfront payment of EUR 6.8 million (USD 8 million) for the acquisition of PXL065 (DRX-065), a clinical-stage program for NASH, and additional programs, including deuterated drug candidates for metabolic, specialty and rare diseases. The company's cash runway extends into 2021 and includes the completion of clinical proof-of-concept studies for NASH for both PXL770 and PXL065.

Poxel reported revenues of EUR 17.5 million for the quarter ended September 30, 2018 and EUR 55.0 million for the nine months ended September 30, 2018 compared with no revenue during the same periods in 2017.

EUR millions	Q1 2018	Q2 2018	Q3 2018	**Sept 2018 9 months**	Q1 2017	Q2 2017	Q3 2017	**Sept 2017 9 months**
Roivant Agreement	8.1	-	-	8.1	-	-	-	**-**
Sumitomo Agreement	10.2	19.2	17.5	46.9	-	-	-	**-**
Total revenues	18.3	19.2	17.5	55.0	-	-	-	**-**

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 and the USD 35 million (EUR 28 million) upfront payment associated with the corporate partnership announced with Roivant Sciences on February 12, 2018 net of Poxel's financial contribution to Roivant. In addition, the revenue also reflects the Imeglimin Phase 3 program costs in Japan incurred during the first nine months of 2018 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.

“I am very pleased to report that during the third quarter we continued to make substantial progress for Imeglimin in Japan and all three pivotal Phase 3 TIMES trials are now fully enrolled with over 1,100 patients. We are on track for the data readout in 2019, beginning with the TIMES 1 efficacy study readout during the second quarter of 2019. We are continuing to work closely with our partner Sumitomo Dainippon Pharma in preparing for the Japanese New Drug Application submission in 2020,” said Thomas Kuhn, CEO of Poxel. “For the United States and Europe, we are working closely with Roivant Sciences and Metavant, a company formed by Roivant Sciences to develop innovative therapies for metabolic disorders, on progressing the Imeglimin clinical program, which is initially targeting patients with type 2 diabetes and moderate-to-severe chronic kidney disease (CKD stages 3b/4), and includes a dedicated clinical trial currently ongoing.”

“For our second program, PXL770, we have also continued to make substantial progress and completed the Phase 1 multiple ascending dose study. We believe that PXL770 has the potential to treat liver diseases, such as NASH, and could have the potential to treat additional metabolic diseases. We are currently preparing for the initiation of a Phase 2a proof-of-concept program in nonalcoholic fatty liver disease (NAFLD) patients who likely have NASH,” added Thomas Kuhn. “With our recent acquisition of DeuteRx’s drug candidate, PXL065 (formerly DRX-065), we are rapidly expanding our presence in NASH and are one of only a few biotech companies with two clinical programs in development in this therapeutic area. The underlying pathophysiological mechanisms that contribute to the development and progression of NAFLD and NASH are highly complex and support the need for the development of novel therapies acting on different targets. Both of our programs, which we plan on advancing into Phase 2 in 2019, have the potential to be developed as a monotherapy or in combination together or with other agents.”

Planned Presentations and Participation at the Following Upcoming Events
BIO Investor, October 17, 2018, San Francisco
2nd Annual NASH Summit Europe, October 22-24, 2018, Frankfurt
American Association for the Study of Liver Diseases, November 9-13, 2018, San Francisco
Jefferies London Healthcare Conference, November 14-15, 2018, London

Next financial press release: Fourth Quarter 2018 Financial Update on February 12, 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 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 (formerly DRX-065)
PXL065 is deuterium-stabilized R-pioglitazone developed by DeuteRx LLC. 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 (stereoisomers) that interconvert *in vivo*. Using deuterium, DeuteRx 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 Trials of IMeglimin for Efficacy and Safety (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 advancing into 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), 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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