August 8, 2022

Poxel Provides Corporate Update and Reports Cash and Revenue for the Second Quarter and First Half 2022

- **Cash runway extended through at least February 2023 based upon debt restructuring agreement with IPF Partners (IPF) and equity-linked financing facility with Iris Capital Investment (IRIS)**
- **As of June 30, 2022, cash and cash equivalents were EUR 16.1 million (USD 16.8 million)**
- **Fast Track and Orphan Drug Designation for PXL065 and PXL770 in adrenoleukodystrophy (ALD) granted by the Food and Drug Administration (FDA)**
- **New Solid Form Patent for PXL065 issued by the U.S. Patent and Trademark Office (PTO), providing additional protection through 2041**
- **Phase 2 study results for PXL065 (DESTINY-1) in NASH 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 metabolic disorders, today provided a corporate update and announced its cash position and revenue for the second quarter and first half of 2022.

"The next major milestone for Poxel will be the results of our Phase 2 DESTINY-1 study for PXL065 in NASH, which are expected later this quarter. One of our key objectives these past months has been to extend our cash runway to leverage this opportunity and independently finance our strategy in rare diseases. The two agreements announced today, the debt restructuring along with the equity-linked financing facility, provide further flexibility to finalize additional financing initiatives, including ongoing active partnership discussions related to our programs. In addition, we will continue our work to initiate our Phase 2 proof-of-concept studies in adrenoleukodystrophy which represent the foundation of our rare disease strategy," **said Thomas Kuhn, Chief Executive Officer of Poxel.** *"We have also had key regulatory achievements this year, including Fast Track Designation and Orphan Drug Designation granted by the FDA for PXL065 and PXL770 in ALD. In addition, the recent patent approval of a new solid form of PXL065 is an important addition to the protection of this compound and significantly extends its exclusivity."*

Commercial Update

TWYMEEG® (Imeglimin)

- As of June 30, 2022, royalty revenue to Poxel based on TWYMEEG net sales in Japan under the Sumitomo Pharma license agreement has been limited following TWYMEEG's commercial launch on September 16, 2021. TWYMEEG's initial commercial uptake has been affected by restrictions in Japan on prescribing any new drug in its first year of commercialization, and conditions related to COVID-19, which have reduced the frequency of physician visits and limited the extensive prescriber education efforts required for any launch of an innovative drug with a new mechanism of action. However, as a result of Sumitomo Pharma's promotional activities and efforts since launch, TWYMEEG is very well known among prescribers.

Clinical Updates

NASH

- PXL065 (deuterium-stabilised R-pioglitazone) is in a Phase 2 study (DESTINY-1). Results from this 36-week, randomized, double-blind, placebo-controlled, parallel group, dose-ranging study designed to assess efficacy and safety are anticipated, as planned, in Q3 2022. The goal of DESTINY-1 is to identify the optimal dose or doses of PXL065 to advance into a Phase 3 registration trial for the treatment of noncirrhotic biopsy-proven NASH patients.

Rare metabolic diseases

- In ALD, two Phase 2a biomarker proof-of-concept (POC) clinical trials of PXL065 and PXL770 are expected to initiate as soon as possible, subject to additional financing. These two identical studies will enroll adult male patients with adrenomyeloneuropathy (AMN), the most common ALD subtype. The POC studies will evaluate the pharmacokinetics, safety and efficacy of PXL065 and PXL770 after 12 weeks of treatment based on relevant disease biomarkers, such as the effect on very long chain fatty acids (VLCFA), the characteristic plasma marker of the disease.
- In February and April, the FDA awarded Fast Track Designation (FTD) to PXL065 and PXL770 respectively, for ALD. The FDA grants FTD to investigational drugs which treat a serious or life-threatening condition, and which fill an unmet medical need. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially better than available therapy. The key benefits of FTD comprise enhanced access to the FDA, with regular and more frequent opportunities for consultation and discussion.
- In May, the FDA granted Orphan Drug Designation (ODD)[1] to PXL065 and PXL770 for ALD. ODD confers a company a potential seven-year window of exclusive marketing rights following FDA approval, along with a reduction in certain application fees, and tax credits for expenses related to qualified clinical trials conducted after orphan designation is received.
- Two preclinical articles on X-Linked Adrenoleukodystrophy (ALD) for PXL065 and PXL770 were published:
    - The article on PXL065 was published in *The Journal of Inherited Metabolic Disease ("JIMD")* and is entitled "*Therapeutic potential of deuterium-stabilized (R)-pioglitazone - PXL065 - for X-linked adrenoleukodystrophy*". It is available here: https://pubmed.ncbi.nlm.nih.gov/35510808/.
    - The article on PXL770 was published in *The Journal of Pharmacology and*

Experimental Therapeutics ("JPET"), and is entitled "*Beneficial effects of the direct AMP-Kinase activator PXL770 in in vitro and in vivo models of X-Linked Adrenoleukodystrophy".* It is available here: https://jpet.aspetjournals.org/content/early/2022/06/25/jpet.122.001208.

Corporate Update

- In June, the U.S. Patent and Trademark Office (PTO) issued a new patent for PXL065 that describes a specific form of PXL065 with unique properties. Importantly, this recently issued patent provides additional protection through 2041 and could expand protection for PXL065 worldwide, with the potential for an additional 5 years through patent term extension.
- On June 21, 2022, 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.
- The mandates of Mrs. Janice Bourque and of Mr. Pierre Legault as members of the Board of Directors were renewed for three year terms. Effective July 1, 2022, Dr. John Kozarich transitioned off as a Board member due to the age limitation and will continue to assist the Board of Directors as a consultant and chair of the scientific committee of the Board.

Significant Events after the Period

The Company announced today that it has entered into an agreement with IPF to restructure its debt, resulting in the postponement of the Q3 2022 and Q4 2022 amortization payments under the existing debt facility, and lowering certain financial covenants until the end of January 2023.

Concurrently, the Company has entered into an equity-linked financing arrangement with IRIS for an initial gross amount of EUR 4 million, with the option, at the latest on December 31, 2022 and, at the Company's sole discretion, to draw a second and third tranche of up to EUR 1 million each. As a result of these two agreements, the Company's expects that its resources will be sufficient to fund its operations and capital expenditure requirements through at least February 2023.

For more information, please refer to the press release issued today.

Second Quarter and First Half 2022 Cash and Cash equivalents

As of June 30, 2022, cash and cash equivalents were EUR 16.1 million (USD 16.8 million), as compared to EUR 32.3 million (USD 36.6 million) as of December 31, 2021.

Net financial debt (excluding IFRS16 impacts and derivative debts) was EUR 17.3 million as of June 30, 2022, as compared to EUR 2.6 million as of December 31, 2021.

EUR (in thousands)	**Q2 2022**	**Q4 2021**
Cash	16,143	28,753

Cash equivalents	-	3,534
Total cash and cash equivalents*	**16,143**	**32,287**

Unaudited data
** Net financial debt (excluding IFRS 16 impacts and derivative debts) was EUR 17.3 million at the end of Q2 2022 as compared to EUR 2.6 million at the end of Q4 2021.*

Based on:

i. its cash position at June 30, 2022,
ii. the current development plan of the Company including 1) the completion of its ongoing Phase 2 NASH trial for PXL065 (DESTINY-1) but excluding 2) the two identical Phase 2a clinical proof-of-concept (POC) biomarker studies for PXL065 and PXL770 in adrenomyeloneuropathy (AMN),
iii. the cash forecast for the year 2022 approved by the Board of Directors of the Company, that does not include, as a conservative approach, any net royalties from Imeglimin in Japan,
iv. a strict control of its operating expenses, and
v. the amendment to the IPF debt facility with the postponement of the Q3 2022 and Q4 2022 amortization payments until end of February 2023, as well as a full drawdown of all tranches of the equity-linked financing arrangement with IRIS for a total amount of EUR 6 million, before December 31, 2022,

the Company expects that its resources will be sufficient to fund its operations and capital expenditure requirements through at least February 2023.

The Company is actively pursuing additional financing options, prioritizing non-dilutive sources, including ongoing active partnership discussions related to its programs.

Second Quarter and First Half 2022 Revenue

Poxel reported revenues of EUR 83 thousand revenue for the six months ended June 30, 2022, as compared to EUR 13.3 million revenue during the corresponding period in 2021.

Revenue for the first half of 2022 reflects JPY 11 million (EUR 81 thousand) of royalty revenue from Sumitomo Pharma which represents 8% of TWYMEEG net sales in Japan. Based on the current forecast, Poxel expects to receive 8% royalties on TWYMEEG net sales in Japan through the Sumitomo Pharma fiscal year 2022 (April 2022 to March 2023). As part of the Merck Serono licensing agreement, Poxel will pay Merck Serono a fixed 8% royalty based on the net sales of Imeglimin, independent of the level of sales.

EUR (in thousands)	**Q1 2022**	**Q2 2022**	**H1 2022**	**Q1 2021**	**Q2 2021**	**H1 2021**
	3 months	**3 months**	**6 months**	**3 months**	**3 months**	**6 months**
Sumitomo Pharma Agreement	32	51	83	-	13,274	13,274
Other	-	-	-	-		

Total revenues	32	51	83	-	13,274	13,274

Unaudited data

Planned Presentations and Participation at the Following Upcoming Events

- Keystone Symposia, Whistler, British Columbia, Canada, August 7-11
- Paris NASH Meeting, Paris, France, September 8-9
- Eurotox, International Congress of Toxicology, Maastricht, Netherlands, September 18-21

Next Financial Press Release : First Half 2022 financial results on September 21, 2022

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 thiazolidinedione (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-1). **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. For 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 and launched for the treatment of type 2 diabetes in Japan. Poxel expects to receive royalties and sales-based payments from Sumitomo Pharma. Poxel has a strategic partnership with Sumitomo 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. The Company does not endorse or is not otherwise responsible for the content of external hyperlinks referred to in this press release.

[1] For more information on Orphan Drug Designation, see: https://www.fda.gov/industry/developing-products-rare-diseases-conditions/designating-

orphan-product-drugs-and-biological-products

View source version on businesswire.com:
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