May 26, 2025

Poxel Announces Positive Preclinical Data for PXL065 in Hypertrophic Cardiomyopathy To Be Presented at the ESC Congress 2025

- **PXL065 demonstrated significant benefits in a HCM mouse model preventing pathological myocardial remodeling, including hypertrophy and fibrosis in the heart**
- **Presentation of detailed results from preclinical study on Sept 1st, 2025, 11:15 am CEST, at the European Society of Cardiology 2025**
- **Findings support the clinical development of PXL065 as a potential disease-modifying treatment for symptomatic and asymptomatic HCM patients**

LYON, France--(BUSINESS WIRE)-- Regulatory News:

POXEL SA (Euronext : POXEL - FR0012432516), a clinical stage biopharmaceutical company developing innovative treatments for chronic serious diseases with metabolic pathophysiology, including metabolic dysfunction-associated steatohepatitis (MASH) and rare metabolic disorders, today announces that the abstract featuring previously announced preclinical data demonstrating positive results for PXL065, the deuterium-stabilized R-enantiomer of pioglitazone, in hypertrophic cardiomyopathy[1], has been accepted for presentation at the 2025 edition of the European Society of Cardiology (ESC) Congress (link), to be held jointly with World Congress of Cardiology, on September 1st, 2025 at 11:15 am CEST, in Madrid, Spain.

Prof. Dr. Cordula Wolf, Director of the Center for Rare Congenital Heart Diseases at the TUM University Hospital German Heart Center, stated: *"The compelling results obtained in this study illustrate the potential of PXL065 in HCM, the most common genetic cardiac disorder. Current treatments have important limitations in efficacy, safety, or patient applicability. There is a clear unmet need for safe and effective disease-modifying therapies."*

Thomas Kuhn, CEO of Poxel, added: *"We are pleased to have the data with PXL065 in HCM be presented at one of the world's leading forums for cardiovascular science and medicine, which underscores both the quality and relevance of these findings. We look forward to further supporting PXL065 development for the treatment of HCM based on these promising results."*

Hypertrophic Cardiomyopathy (HCM) is a genetic disorder marked by myocardial

hypertrophy, cardiac fibrosis, ventricular dysfunction, arrhythmias, and an increased risk of sudden cardiac death. It is caused by mutations in sarcomere protein genes, leading to altered cell metabolism, including oxidative stress and mitochondrial dysfunction. The estimated prevalence of HCM is 0.2%, or 1/500 adults, and its incidence is around 5 per 100,000 person-years.

In connection with the mechanism of action of PXL065 on the inhibition of the mitochondrial pyruvate carrier (MPC) and on the inhibition of Acyl CoA Synthetase 4 (ACSL4) thus acting on oxidative stress, inflammation and fibrosis, PXL065 was successfully assessed in an established mouse model of hypertrophic cardiomyopathy.

This preclinical study was funded by the German Center for Cardiovascular Research (DZHK) and conducted at the TUM University Hospital German Heart Center by leading HCM expert Prof. Dr. Cordula Wolf. Poxel and the TUM University Hospital German Heart Center collaborated on the pre-clinical study based on Poxel's existing data and patent portfolio on PXL065 and prior research conducted by Prof. Dr. Cordula Wolf and her group on the disease mechanisms and therapeutic use of TZD's in HCM.

About Poxel SA

Poxel is a **clinical stage biopharmaceutical company** developing **innovative treatments for chronic serious diseases with metabolic pathophysiology**, including **metabolic dysfunction-associated steatohepatitis (MASH)** and rare disorders. For the treatment of MASH, **PXL065** (deuterium-stabilized *R*-pioglitazone) met its primary endpoint in a streamlined Phase 2 trial (DESTINY-1). In rare diseases, development of **PXL770**, a first-in-class direct adenosine monophosphate-activated protein kinase (AMPK) activator, is focused on the treatment of adrenoleukodystrophy (ALD) and autosomal dominant polycystic kidney disease (ADPKD). **TWYMEEG®** (Imeglimin), Poxel's first-in-class product that targets mitochondrial dysfunction, is now marketed for the treatment of type 2 diabetes in Japan by Sumitomo Pharma and Poxel expects to receive royalties and sales-based payments. Poxel has a strategic partnership with Sumitomo Pharma for Imeglimin in Japan. 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

[1] *Press release as of March 20, 2025*

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