Pharming announces enrolment of first patient in multicentre Phase IIb clinical trial of RUCONEST® for the prevention of acute kidney injury after myocardial infarction

Highlights:

- Clinical trial follows encouraging results from a Phase II, which showed a statistically significant improvement of kidney injury biomarkers in patients undergoing coronary angiography after treatment with RUCONEST®
- First patient has been enrolled at the University Hospital Basel, Switzerland, with additional clinical centres in Switzerland to join the study

Leiden, The Netherlands, 22 April 2021*:* Pharming Group N.V. ("Pharming" or "the Company") (Euronext Amsterdam: PHARM/Nasdaq: PHAR) announces that the first patient has been enrolled in a Phase IIb double-blind, randomized, controlled study to assess the efficacy of RUCONEST® (recombinant human C1 esterase inhibitor, or "rhC1INH") for the prevention of acute kidney injury after non-ST elevation myocardial infarction at the University Hospital Basel, Switzerland.

In October 2018, Pharming Group N.V. announced positive results from a Phase II investigator-initiated, double-blind, placebo-controlled clinical trial of RUCONEST® in patients at risk of nephropathy following coronary angiography. The positive results were especially clear in the sub-group of patients undergoing percutaneous coronary interventions such as stent insertions. The intent-to-treat analysis in this group showed that patients on RUCONEST® had a median percentage change in peak urinary neutrophil gelatinase-associated lipocalin within 48 hours, the primary endpoint for the study and a generally-recognized early marker of acute renal injury, of 11.3% in the RUCONEST® arm and 205.2% in the placebo arm (p=0.001). The overall assessment of the study also showed trends that patients undergoing more invasive interventions and procedures requiring higher volumes of contrast medium experienced a stronger benefit from treatment with RUCONEST®.

Following these encouraging results, the Company, in partnership with treating physician Dr Michael Osthoff from the University Hospital of Basel, Switzerland, saw potential for a larger, randomized, controlled multicenter study to investigate the full extent of the role of RUCONEST® in the prevention of acute kidney damage after percutaneous coronary intervention for myocardial infarction. If successful, the clinical trial could also support further development of RUCONEST® to prevent AKI from a wide variety of causes.

Prof. Bruno Giannetti, Pharming's Chief Medical Officer, commented:

"We are excited to initiate this important study with RUCONEST® and look forward to a swift recruitment rate. The design of this clinical trial allows us to assess important NGAL levels, as well as a large number of clinical and laboratory parameters, which will provide valuable information in, not only, the assessment of acute kidney injury in the setting of myocardial infarction, but eventually across even broader etiologies. If found to be efficacious, RUCONEST® could make a significant contribution to improving the life expectancy and quality of life of patients suffering from kidney damage."

About the study

The double-blind, randomized, controlled study to assess the efficacy of RUCONEST® for the prevention of acute kidney injury after non-ST elevation myocardial infarction (NSTEMI) will include up to 220 patients. The primary end point is to evaluate the efficacy of rhC1INH compared to placebo after

Percutaneous Coronary Intervention (PCI) in NSTEMI patients by examining the peak increase of urinary NGAL (Urinary neutrophil gelatinase-associated lipocalin) within 24 hours following treatment. In addition, the study will seek to identify an appropriate dosing regimen for potential future studies. Additional end points for the study include the incidence of AKI defined by serum creatinine increase within 72 hours after angiography, as well as cardiovascular and renal events and hospitalization-related medical resource utilization for 6 months. The study will be conducted in various centres across Switzerland.

About acute kidney injury

Acute Kidney Injury (AKI) affects approximately 20% of all patients admitted to hospital. It is defined by rapid onset of renal damage and dysfunction. It is an important complication that can impact both short- and long-term patient outcomes. Its incidence may increase to >50% in seriously ill patients or in the presence of risk factors such as chronic kidney disease, diabetes mellitus and nephrotoxic drugs. In the setting of myocardial infarction, AKI can be caused by multiple mechanisms including nephrotoxicity of the contrast agent as well as ischemia-reperfusion injury following PCI.

About Pharming Group N.V.

Pharming Group N.V. is a global, commercial stage biopharmaceutical company developing innovative protein replacement therapies and precision medicines for the treatment of rare diseases and unmet medical needs.

The flagship of our portfolio is our recombinant human C1 esterase inhibitor (rhC1INH) franchise. C1INH is a naturally occurring protein that down regulates the complement and contact cascades in order to control inflammation in affected tissues.

Our lead product, RUCONEST®, is the first and only plasma-free rhC1INH protein replacement therapy. It is approved for the treatment of acute hereditary angioedema (HAE) attacks. We are commercializing RUCONEST® in the United States, the European Union and the United Kingdom through our own sales and marketing organization, and the rest of the world through our distribution network.

In addition, we are investigating the clinical efficacy of rhC1INH in the treatment of further indications, including pre-eclampsia, acute kidney injury and severe pneumonia as a result of COVID-19 infections.

We are also studying our oral precision medicine, leniolisib (a phosphoinositide 3-kinase delta, or PI3K delta, inhibitor), for the treatment of activated PI3K delta syndrome, or APDS, in a registration enabling Phase 2/3 study in the United States and Europe.

Furthermore, we are leveraging our transgenic manufacturing technology to develop next-generation protein replacement therapies, most notably for Pompe disease, which is currently in preclinical development.

For more information, please visit www.pharming.com

Forward-looking Statements

This press release contains forward-looking statements, including with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, Pharming's ability to overcome the challenges posed by the COVID-19 pandemic to the conduct of its business, and Pharming's expectations regarding its projected working capital requirements and cash resources, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope, progress and expansion of Pharming's clinical trials and ramifications for the cost thereof; and clinical, scientific, regulatory and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that that are described in Pharming's 2020 Annual Report and the Annual Report on Form 20-F for the year ended December 31,

2020 filed with the U.S. Securities and Exchange Commission, the events and circumstances discussed in such forward-looking statements may not occur, and Pharming's actual results could differ materially and adversely from those anticipated or implied thereby. Any forward-looking statements speak only as of the date of this press release and are based on information available to Pharming as of the date of this release.

Inside Information

This press release relates to the disclosure of information that qualifies, or may have qualified, as inside information within the meaning of Article 7(1) of the EU Market Abuse Regulation.

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