

# Pharming announces the publication of data from a compassionate use programme of RUCONEST<sup>®</sup> in COVID-19 patients in a peerreviewed journal

*Leiden, The Netherlands,* **17** August 2020: Pharming Group N.V. ("Pharming" or "the Company") (Euronext Amsterdam: PHARM) today announces the publication of data in the peer-reviewed journal, Frontiers in Immunology, from a compassionate use programme of five patients with confirmed COVID-19 (SARS-CoV-2) infections hospitalised with related severe pneumonia that were treated with RUCONEST<sup>®</sup> (recombinant human C1 inhibitor, conestat alfa) at the University Hospital Basel, Switzerland.

As reported on 21 April 2020, following treatment with RUCONEST<sup>®</sup>, fever resolved in four of the five patients within 48 hours, and laboratory markers of inflammation decreased significantly (CRP, IL-6). Soon thereafter, four of the five patients were discharged from the hospital as fully recovered. One patient had increased oxygen requirement and was temporarily transferred to the ICU for intubation but over the subsequent days made a full recovery.

Given this was a small case series, the outcomes were retrospectively compared to a matched control population of 15 patients. Baseline characteristics, admission laboratory parameters and treatments administered were similar in both groups. Both groups received standard of care as well as experimental therapies including antiviral and anti-cytokine directed medications. However, 8/15 (53%) patients in the control population required mechanical ventilation and 4 of these patients died, compared to only 1 (20%) requiring mechanical ventilation and no deaths in the RUCONEST<sup>®</sup> group. Overall, treatment with, in total five normal dose equivalents of RUCONEST<sup>®</sup> over 48h was well-tolerated.

The full publication is available on the company's website <u>www.pharming.com</u>

## About Pharming Group N.V.

Pharming is a specialty pharmaceutical company developing innovative products for the safe, effective treatment of rare diseases and unmet medical needs. Pharming's lead product, RUCONEST<sup>®</sup> (conestat alfa) is a recombinant human C1 esterase inhibitor approved for the treatment of acute attacks of angio-edema in patients with hereditary angioedema (HAE) in Europe; for adults, adolescents and children from two years of age, in the US, for adults and adolescents and in Israel and South Korea. The product is available on a named-patient basis in other territories where it has not yet obtained marketing authorisation.

RUCONEST<sup>®</sup> is commercialised by Pharming in the US and in Europe, and the Company holds all other commercialisation rights in other countries not specified below. In some of these other countries distribution is made in association with the HAEi Global Access Program (GAP). RUCONEST<sup>®</sup> is distributed in Argentina, Colombia, Costa Rica, the Dominican Republic, Panama, and Venezuela by Cytobioteck, in South Korea by HyupJin Corporation and in Israel by Kamada.

RUCONEST<sup>®</sup> is also being evaluated for various additional indications. Pharming's technology platform includes a unique production process that has proven capable of producing industrial quantities of



pure high quality recombinant human proteins in a more economical and less immunogenic way compared with current cell-line based methods.

Leads for enzyme replacement therapy ("ERT") for Pompe and Fabry's diseases are also being produced and optimised respectively at present.

Pharming has recently in-licensed leniolisib from Novartis, a small molecule and selective PI3K $\delta$  inhibitor, which is in a registrational study for activated PI3K-delta syndrome (APDS), a rare form of Primary Immunodeficiency.

Pharming has a long-term partnership with the China State Institute of Pharmaceutical Industry ("CSIPI"), a Sinopharm company, for joint global development of new products, starting with recombinant human Factor VIII for the treatment of Haemophilia A. Preclinical development and manufacturing will take place to global standards at CSIPI and its affiliates and are funded by CSIPI. Clinical development will be shared between the partners with each partner taking the costs for their territories under the partnership.

## Forward-looking Statements

This press release of Pharming Group N.V. and its subsidiaries ("Pharming", the "Company") may contain forward-looking statements including without limitation those regarding Pharming's financial projections, market expectations, developments, partnerships, plans, strategies and capital expenditures.

The Company cautions that such forward-looking statements may involve certain risks and uncertainties, and actual results may differ. Risks and uncertainties include without limitation the effect of competitive, political and economic factors, legal claims, the Company's ability to protect intellectual property, fluctuations in exchange and interest rates, changes in taxation laws or rates, changes in legislation or accountancy practices and the Company's ability to identify, develop and successfully commercialise new products, markets or technologies.

As a result, the Company's actual performance, position and financial results and statements may differ materially from the plans, goals and expectations set forth in such forward-looking statements. The Company assumes no obligation to update any forward-looking statements or information, which should be taken as of their respective dates of issue, unless required by laws or regulations.

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