

Pharming announces positive data from paediatric clinical trial with RUCONEST®

RUCONEST® provided clinically meaningful relief of symptoms in children with hereditary angioedema

Leiden, The Netherlands, 02 October 2017: Pharming Group N.V. (“Pharming” or “the Company”) (Euronext Amsterdam: PHARM) announced today positive data from a clinical trial with the use of RUCONEST® [Recombinant Human C1 Esterase Inhibitor/ conestat alfa] for the treatment of hereditary angioedema (HAE) attacks in children.

The open-label, single arm, Phase II clinical trial was designed in agreement with the European Medicines Agency (EMA) as part of a Paediatric Investigation Plan (PIP) to assess the pharmacokinetic, safety and efficacy profiles of RUCONEST® at a dose of 50 IU/kg in paediatric HAE patients ages 2-13 years in support of a paediatric indication for treatment of HAE attacks.

A total of 20 children with HAE were treated for 73 HAE attacks at a dose of 50 IU/kg (up to a maximum of 4200 IU). The study reported clinically meaningful relief of symptoms assessed using a visual analogue scale (VAS) completed by the patient (assisted by their parent). The median time to onset of relief was 60 minutes (95% confidence interval: 60-63), and the median time to minimal symptoms was 122 minutes (95% confidence interval: 120-126). Only 3/73 (4%) attacks were treated with a second dose of RUCONEST®.

RUCONEST® was generally safe and well-tolerated in the study. No patients withdrew from the study due to adverse events. There were no related serious adverse events, hypersensitivity reactions, or neutralizing antibodies detected.

The detailed efficacy and safety results will be further analysed and submitted for presentation at an upcoming major medical meeting.

Prof. Bruno Giannetti, Pharming’s Chief Operations Officer, commented:

“The results of this study provide important safety and efficacy data on the use of RUCONEST® in children and show good clinical concordance with results from adolescents and adults with HAE. Children with HAE have limited therapeutic options, and we will work expeditiously with regulatory authorities to expand choices for these patients.”

– ENDS –

Forward-looking Statements

This press release of Pharming Group N.V. and its subsidiaries (“Pharming”, the “Company” or the “Group”) 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 commercialize 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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About HAE

Hereditary Angioedema (HAE) is a rare genetic disorder. It is characterized by spontaneous and recurrent episodes of swelling (edema attacks) of the skin in different parts of the body, as well as in the airways and internal organs. Edema of the skin usually affects the extremities, the face, and the genitals. Patients suffering from this kind of edema often withdraw from their social lives because of the disfiguration, discomfort and pain these symptoms may cause. Almost all HAE patients suffer from bouts of severe abdominal pain, nausea, vomiting and diarrhoea caused by swelling of the intestinal wall.

Edema of the throat, nose or tongue can be particularly dangerous as this can lead to obstruction of the airway passages and be potentially life threatening. Although there is currently no known cure for HAE, it is possible to treat the symptoms associated with edema attacks. HAE affects about 1 in 10,000 to 1 in 50,000 people, worldwide experts believe that a lot of patients are still seeking the right diagnosis: although HAE is (in principle) easy to diagnose, it is frequently identified very late or not discovered at all. The reason HAE is often misdiagnosed is because the symptoms are similar to those of many other common conditions such as allergies or appendicitis by the time it is diagnosed correctly, the patient has often been through a long-lasting ordeal.

About RUCONEST®

US INDICATION

RUCONEST® (C1 esterase inhibitor [recombinant]) is indicated for the treatment of acute attacks in adult and adolescent patients with hereditary angioedema (HAE). Effectiveness in clinical studies was not established in HAE patients with laryngeal attacks.

IMPORTANT SAFETY INFORMATION

- RUCONEST® (C1 esterase inhibitor [recombinant]) is contraindicated in:
 - Patients with a history of allergy to rabbits or rabbit-derived products.
 - Patients with a history of life-threatening immediate hypersensitivity reactions to C1 esterase inhibitor preparations, including anaphylaxis.
- **Hypersensitivity:** Severe hypersensitivity reactions may occur. Should symptoms occur, discontinue RUCONEST and administer appropriate treatment. Because hypersensitivity reactions may have symptoms similar to HAE attacks, treatment methods should be carefully considered.
- **Thromboembolic Events:** Serious arterial and venous thromboembolic (TE) events have been reported at the recommended dose of plasma-derived C1 esterase inhibitor products in patients with risk factors. Risk factors may include the presence of an indwelling venous catheter/access device, prior history of thrombosis, underlying atherosclerosis, use of oral contraceptives or certain androgens, morbid obesity, and immobility. Monitor patients with known risk factors for TE events during and after RUCONEST administration.
- **Intravenous Use:** RUCONEST is for intravenous use after reconstitution only. No more than 2 doses should be administered within a 24-hour period.
- **Pregnancy and Nursing:** RUCONEST has not been studied in pregnant women; therefore, it should only be used during pregnancy if clearly needed. Advise patients to notify their physician if they are breastfeeding or plan to breastfeed.
- **Adverse reactions:** The serious adverse reaction in clinical studies of RUCONEST was anaphylaxis.
- **Common adverse reactions:** The most common adverse reactions (incidence $\geq 2\%$) were headache, nausea, and diarrhea.

Please see Full Prescribing Information for RUCONEST® as applicable for various jurisdictions:

[FDA: RUCONEST®¹](#) / [EMA: RUCONEST®²](#)

¹<https://www.fda.gov/downloads/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/LicensedProductsBLAs/FractionatedPlasmaProducts/UCM405634.pdf>

²http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Product_Information/human/001223/WC500098542.pdf

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® (conestat alfa) is a recombinant human C1 esterase inhibitor approved for the treatment of acute Hereditary Angioedema ("HAE") attacks in patients in Europe, the US, Israel and South Korea. The product is available on a named-patient basis in other territories where it has not yet obtained marketing authorization.

RUCONEST® is commercialized by Pharming in Algeria, Andorra, Austria, Bahrain, Belgium, France, Germany, Ireland, Jordan, Kuwait, Lebanon, Luxembourg, Morocco, the Netherlands, Oman, Portugal, Qatar, Syria, Spain, Switzerland, Tunisia, the United Arab Emirates, the United Kingdom, the United States of America and Yemen.

RUCONEST® is distributed by Swedish Orphan Biovitrum AB (publ) (SS: SOBI) in the other EU countries, and in Azerbaijan, Belarus, Georgia, Iceland, Kazakhstan, Liechtenstein, Norway, Russia, Serbia and Ukraine.

RUCONEST® 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 Megapharm.

RUCONEST® has recently completed a clinical trial for the treatment of HAE in young children (2-13 years of age) and is also evaluated for various additional follow-on indications.

Pharming's technology platform includes a unique, GMP-compliant, validated process for the production of pure recombinant human proteins that has proven capable of producing industrial quantities of high quality recombinant human proteins in a more economical and less immunogenetic way compared with current cell-line based methods. Leads for enzyme replacement therapy ("ERT") for Pompe and Fabry's diseases are being optimized at present, with additional programs not involving ERT also being explored at an early stage at present.

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. Pre-clinical development and manufacturing will take place to global standards at CSIPI 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.

Pharming has declared that the Netherlands is its "Home Member State" pursuant to the amended article 5:25a paragraph 2 of the Dutch Financial Supervision Act.

Additional information is available on the Pharming website: www.pharming.com