Pharming Announces Positive Results from Randomized Controlled Trial with RUCONEST® for HAE Prophylaxis

RUCONEST® showed clinically and statistically significant reduction in attack frequency both in twice weekly and once- weekly dosing

Leiden, The Netherlands, 18 July 2016: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT: PHARM) today announced positive results from a Phase 2 clinical study of RUCONEST® (recombinant C1 esterase inhibitor, 50 IU/kg) for prophylaxis in patients with hereditary angioedema (HAE). In the study, RUCONEST® showed a clinically relevant and statistically significant reduction in attack frequency for both the twice-weekly and once-weekly treatment regimens as compared with placebo.

Thirty-two HAE patients deficient in C1 esterase inhibitor and with a history of at least four attacks per month were enrolled in the randomized, double-blind, placebo-controlled study. The patients received RUCONEST® once and twice weekly and placebo in each of three four-week treatment periods in a cross-over design. The primary efficacy endpoint was the number of HAE attacks per 28 day treatment period and the secondary endpoint was clinical response, defined as a ≥ 50% reduction in the number of attacks from treatment with placebo to treatment with RUCONEST®.

In the intent-to-treat analysis (ITT), the study found a statistically significant difference in the mean number of HAE attacks that patients experienced during treatment with both the twice-weekly (p-value <0.0001) and once-weekly (p-value =0.0004) RUCONEST® regimen as compared with placebo.

Patients on placebo had a mean of 7.2 attacks (95% confidence interval[CI]: 5.8-8.6) per four week treatment period which was reduced to a mean of 2.7 attacks on RUCONEST® twice weekly (95% CI: 1.8-3.7) and a mean of 4.4 attacks on RUCONEST® once-weekly (95% CI: 3.1-5.6).

For the analysis of the secondary endpoint in the ITT population, 74% of patients (95% CI: 57-86) on the twice-weekly RUCONEST® regimen had at least a 50% reduction in their attack frequency.

This was confirmed in the per-protocol population of patients, which included patients who completed the study without any major deviations (n=23), where 96% of patients (95% CI: 79-99) on the twice-weekly RUCONEST® regimen and 57% (95%CI: 37-74) on the once weekly RUCONEST® regimen had at least a 50% reduction in their attack frequency. Furthermore, in this group, twice weekly RUCONEST® treatment reduced the attack frequency by 72% (95% CI: 63-81) and once weekly RUCONEST® treatment reduced attack frequency by 44% (95% CI: 27-62) as compared with placebo.

RUCONEST® was generally well-tolerated in the study. No patients withdrew from the study due to adverse events. There were no related serious adverse events. There were no thrombotic or thromboembolic events observed. There were no hypersensitivity or anaphylactic reactions. There were also no neutralizing antibodies detected.

Marc Riedl, Professor of Medicine and Clinical Director of the US HAEA Angioedema Center at UCSD and co-prinicipal investigator for the study, commented: "The results of this well-controlled prophylactic study demonstrate a clinically relevant reduction of HAE attack frequency and a high

responder rate with the recombinant C1INH treatment. Combined with the excellent safety profile, this data supports further development of recombinant C1INH as a useful preventive therapy for HAE."

Marco Cicardi, Professor of Internal Medicine University of Milan, Hospital L. Sacco Milan and co-prinicipal investigator for the study, remarked: "The clinical efficacy and responder rate in this well-controlled study clearly indicate that, despite its short half life, recombinant C1-inhibitor has the potential to become a prophylactic treatment for HAE. The results also mark an important step forward to further understanding the underlying mode of action of C1-inhibitor therapy in the treatment of HAE."

Prof. Dr. Bruno Giannetti, Pharming's COO, added: "We are very encouraged by these results and now look forward to reviewing the results with the FDA and EMA to be able to determine how to bring RUCONEST®, as the first and only recombinant C1- inhibitor, to patients who need prophylactic treatment for their HAE."

Under the terms of the North American licensing agreement with Valeant Pharmaceuticals International, Valeant and Pharming share 50/50 the development costs for RUCONEST® for prophylaxis of HAE. Pharming will receive an undisclosed milestone payment from Valeant as and when FDA approval for this additional indication is given. RUCONEST® has been granted Orphan Drug designation by FDA for the prophylactic treatment of angioedema caused by hereditary or acquired C1 esterase inhibitor deficiency, with data exclusivity until 2026 under the Biologics Price Competition and Innovation Act.

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Important Safety Information for RUCONEST®

Indication

RUCONEST® is a recombinant C1 esterase inhibitor 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.

RUCONEST (C1 esterase inhibitor [recombinant]) is contraindicated in patients with a history of allergy to rabbits or rabbit-derived products, and patients with a history of life-threatening immediate hypersensitivity reactions to C1 esterase inhibitor preparations, including anaphylaxis.

Severe hypersensitivity reactions may occur. The signs and symptoms of hypersensitivity reactions may include hives, generalized urticaria, tightness of the chest, wheezing, hypotension, and/or anaphylaxis during or after injection of RUCONEST. 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.

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 implanted 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.

RUCONEST has not been studied in pregnant women; therefore, it should only be used during pregnancy if clearly needed.

The most common adverse reactions (incidence ≥2%) were headache, nausea, and diarrhea. The serious adverse reaction in clinical studies of RUCONEST was anaphylaxis.

Please see complete Prescribing Information for RUCONEST.

About RUCONEST®

RUCONEST® (recombinant C1 esterase inhibitor) 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.

HAE is caused by a deficiency of the C1 esterase inhibitor protein, which is present in blood and helps control inflammation (swelling) and parts of the immune system. A shortage of C1 esterase inhibitor can lead to repeated attacks of swelling, pain in the abdomen, difficulty breathing and other symptoms. RUCONEST contains C1 esterase inhibitor at 50 IU/kg.

When administered at the onset of HAE attack symptoms at the recommended dose, RUCONEST may help to return a patient's C1 esterase inhibitor levels to normal range and relieve the symptoms of an HAE attack with a low recurrence of symptoms within 24 hours.

RUCONEST is the only recombinant C1 esterase inhibitor approved by the U.S. Food and Drug Administration (FDA) and was approved in July 2014.

Under the Biologics Price Competition and Innovation Act of 2009, RUCONEST was granted Market exclusivity in the USA until July 2026

RUCONEST is designated as an orphan drug by the FDA for the treatment of acute attacks of angioedema caused by hereditary or acquired C1 esterase inhibitor deficiency.

About HAE

Hereditary Angioedema (HAE) is a rare genetic disorder. It is characterised 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 diarrhea caused by swelling of the intestinal wall.

Edema of the throat, nose or tongue is particularly dangerous and potentially life-threatening and can lead to obstruction of the airway passages. 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 Pharming Group NV

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 and rest of the world. 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 Austria, Germany and The Netherlands.

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 North America, Canada and Mexico by Valeant Pharmaceuticals International, Inc. (NYSE: VRX/TSX: VRX), following Valeant's acquisition of Salix Pharmaceuticals, Ltd.

RUCONEST® is distributed in Argentina, Colombia, Costa Rica, the Dominican Republic, Panama and Venezuela, by Cytobioteck.

RUCONEST® is distributed in South Korea by HyupJin Corporation and in Israel by Megapharm.

RUCONEST® is being investigated in a Phase II randomized, double blind placebo-controlled clinical trial for prophylactic treatment of HAE and is being evaluated for other indications as well.

RUCONEST® is also being investigated in a Phase II clinical trial for the treatment of HAE in young children (2-13 years of age) and 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 at an early stage of development.

Pharming has a long term partnership with the Shanghai Institute of Pharmaceutical Industry ("SIPI"), 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 SIPI and are funded by SIPI. 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

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 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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