

## PHARMING ANNOUNCES ADOPTION BY THE EUROPEAN COMMISSION OF THE CHMP RECOMMENDATION TO CHANGE THE TERMS OF THE MARKETING AUTHORISATION FOR RUCONEST®

### -TREATMENT OF HAE ATTACKS IN ADOLESCENTS WITH HAE APPROVED- -IGE TESTING REQUIREMENTS REMOVED-

*Leiden, The Netherlands, 07 April 2016:* Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT: PHARM) today announced that the European Commission adopted the CHMP recommendation to include the treatment of HAE attacks in adolescents with HAE and to remove the requirements for rabbit IgE testing that formed part of the EU label for RUCONEST®. The CHMP also noted that the importance of favourable effects of RUCONEST® is further supported by the continued availability of supply of RUCONEST® (produced by recombinant technology) in comparison to supply from blood donor plasma that may vary and not being a blood derived product thereby removing the potential risk of exposure to blood borne pathogens.

This will mean that, effective now, adolescents also have access to (non- blood derived) recombinant C1-inhibitor therapy for the treatment of their angioedema attacks. In addition, the requirement to test HAE patients for pre-existing antibodies against rabbit dander, prior to treatment with RUCONEST and following each tenth treatment with RUCONEST, has been removed from the label. The requirement for IgE testing was a specific EU request based on a single adverse drug reaction in a study subject. The need for testing was not required in the US as more safety data were available at the time of the Biologics License Application (BLA) and subsequent FDA-approved label in 2014. The EU patient information leaflet will be updated to reflect these changes over the coming months.

Prof. Bruno Giannetti, MD, PhD, Pharming's COO commented: "This EU label change now also gives adolescents in the EU the long awaited access to treat their HAE attacks with a non- blood derived C1- inhibitor and in addition, the adoption by the European commission of the CHMP recommendation again confirms the well-established safety profile of RUCONEST®, based on a database of a dozen controlled clinical trials as well as now some 13,000 post-marketing doses of RUCONEST® provided to HAE patients. We are pleased that the burden on patients and doctors to perform testing prior to and after treatments with RUCONEST® has been removed and that emergency treatments of HAE attacks in previously untreated HAE patients, that were until now dependent on plasma derived C1INH therapies can now be made with RUCONEST®, with, as per the CHMP opinion, the added benefit of eliminating risks of exposure to known blood borne pathogens, such as Hepatitis A, B, C, E, HIV, and CJD, as well as continuously (re)-emerging other pathogens.

Please see Prescribing Information for RUCONEST as applicable for various jurisdictions.

<http://www.pharming.com/products/ruconest>

<http://www.fda.gov/downloads/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/LicensedProduct sBLAs/FractionatedPlasmaProducts/UCM405634.pdf>

#### 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 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 the EU, Norway, Iceland and Liechtenstein, the USA, Israel and South Korea.

In other territories where it has not yet obtained marketing authorization, RUCONEST® is available through the international HAE patient organization's Global Access Programme (HAEi-GAP) on a named-patient basis.

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 Cytobiotech.

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. The Phase II study was fully recruited shortly after the year-end 2015.

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 being explored at an early stage at present.

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](http://www.pharming.com)

## *Pharming Disclosure Notice*

*This press release contains forward looking statements that involve known and unknown risks, uncertainties and other factors, which may cause the actual results, performance or achievements of the Company to be materially different from the results, performance or achievements expressed or implied by these forward looking statements.*

## **Contacts:**

### **Pharming Group N.V.**

Sijmen de Vries, CEO: T: +31 71 524 7400

### **FTI Consulting:**

Julia Phillips/ Victoria Foster Mitchell, T: +44 203 727 1136