

# PHARMING

---

## PHARMING ANNOUNCES PRESENTATION OF NEW RUCONEST® CLINICAL DATA

### Findings presented at the annual meeting of the European Academy of Allergy and Clinical Immunology

**Leiden, The Netherlands, June 18, 2012.** Biotech company Pharming Group NV ("Pharming" or "the Company") (NYSE Euronext: PHARM) today presented clinical safety and efficacy data for Ruconest® (recombinant human C1 inhibitor, or rhC1INH) at the annual meeting of the European Academy of Allergy and Clinical Immunology (EAACI), Geneva, Switzerland, 16 to 20 June 2012.

The new data covers a number of aspects that are relevant to the increasing number of physicians that use Ruconest in the day-to-day treatment of HAE patients in the European Union: Efficacy and safety data for Ruconest in the treatment of adolescents suffering from acute attacks of HAE; underpinning a potential extension of the European labeling of Ruconest and "real-life" experience by French physicians; building confidence in Ruconest by successful treatment with Ruconest of a HAE patient that previously failed various other treatments.

In addition an analysis of a previously reported open-label study to explore potential benefits of Ruconest in prophylaxis of HAE, potentially an additional indication that could be explored.

The headlines and authors of the poster presentations are:

Toubi *et al*: "Safety and Efficacy evaluation of rhC1INH for the treatment of HAE attacks in adolescent patients". This analysis reviewed data from 16 adolescent HAE patients who were treated for a total of 50 angioedema attacks with Ruconest. Patients were treated up to 7 times for HAE attacks at all locations. Median times to onset of symptom relief for successive attacks ranged from 19 to 123 minutes. Median times to minimal symptoms ranged from 120 to 650 minutes. Ninety percent of the attacks responded within 4 hours after treatment, and none of the attacks relapsed. The most frequently reported adverse event was headache. No hypersensitivity reactions and no drug-related serious adverse events were observed. No treatment-emergent antibodies developed in these patients.

Bouillet *et al*: The case of a type III HAE patient who failed several therapies and was successfully treated with Ruconest.

Reshef *et al*: "Safety and Efficacy of a weekly infusion of Recombinant Human C1 Inhibitor (rhC1INH) for Prophylaxis of Hereditary Angioedema (HAE) attacks". This was an open label study on the prophylactic effect of once-weekly administration of Ruconest in 25 HAE patients. Patients included in this study had a history of frequent HAE attacks, with a significant impact on their quality of life. The frequency of HAE attacks during the 8 week treatment period was reduced by approximately 50 percent, from a median of 0.6 attacks per week to 0.3. The repeated administrations were generally safe and well-tolerated.

### About the EAACI Annual Meeting

The EAACI Congress 2012 will attract around 7000 international clinicians, researchers and allied health professionals. This is the most important professional meeting for advances in research, treatment and prevention of allergic and immunologic diseases (such as asthma, rhinitis, eczema and occupational allergy, food and drug allergy, severe anaphylactic reactions, rheumatic and autoimmune diseases, AIDS). More information on the congress can be found on [www.eaaci2012.com](http://www.eaaci2012.com).

### RUCONEST® Phase III Study

Pharming is conducting a Phase III clinical study with RUCONEST® under a Special Protocol Assessment (SPA) that is intended to support the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration

(FDA). RUCONEST is being evaluated for the treatment of acute attacks of angioedema in patients with HAE in an international, multicenter, randomized, placebo-controlled Phase III study at a dosage of 50 U/kg with a primary endpoint of time to beginning of relief of symptoms. Santarus has licensed certain exclusive rights from Pharming to commercialize RUCONEST in North America for the treatment of acute attacks of HAE and other future indications. Under the terms of the license agreement, a \$10 million milestone is payable to Pharming upon successful achievement of the primary endpoint of the Phase III clinical study. The study is expected to be completed in Q3 2012.

#### **About RUCONEST® and Hereditary Angioedema**

RUCONEST® (INN conestat alfa) is a recombinant version of the human protein C1 inhibitor (C1INH). RUCONEST is produced through Pharming's proprietary technology in milk of transgenic rabbits and is approved in Europe for treatment of acute angioedema attacks in patients with HAE. RUCONEST® is an investigational drug in the U.S. and has been granted orphan drug designation for the treatment of acute attacks of HAE, a genetic disorder in which the patient is deficient in or lacks a functional plasma protein C1 inhibitor, resulting in unpredictable and debilitating episodes of intense swelling of the extremities, face, trunk, genitals, abdomen and upper airway. The frequency and severity of HAE attacks vary and are most serious when they involve laryngeal edema, which can close the upper airway and cause death by asphyxiation. According to the U.S. Hereditary Angioedema Association, epidemiological estimates for HAE range from one in 10,000 to one in 50,000 individuals.

#### **About Pharming Group NV**

Pharming Group NV is developing innovative products for the treatment of unmet medical needs. RUCONEST® is a recombinant human C1 inhibitor approved for the treatment of angioedema attacks in patients with HAE in all 27 EU countries plus Norway, Iceland and Liechtenstein, and is distributed in the EU by Swedish Orphan Biovitrum (OMX: SOBI). RUCONEST® is partnered with Santarus, Inc (NASDAQ: SNTS) in North America where the drug is undergoing Phase III clinical development. The product is also being evaluated for follow-on indications in the areas of transplantation and reperfusion injury. The advanced technologies of the Company include innovative and validated platforms for the production of protein therapeutics, technology and processes for the purification and formulation of these products. A feasibility study, using the validated transgenic rabbit platform, aimed at the development of recombinant Factor VIII for the treatment of Haemophilia A is underway with partner, Renova Life, Inc. Additional information is available on the Pharming website, [www.pharming.com](http://www.pharming.com). To download the Pharming Group Investor Relations App, click [here](#).

*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.*

#### **Contact**

Sijmen de Vries, CEO: T: +31 (0)71 524 7400  
Karl Keegan, CFO: T: +31 (0)71 524 7400

FTI Consulting  
Julia Phillips/ John Dineen, T: +44 (0)207 269 7193

###