

# PHARMING

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## PHARMING ANNOUNCES INITIATION OF A PEDIATRIC PHASE II CLINICAL STUDY WITH RUCONEST

### Discontinues antibody-mediated rejection Phase II clinical study

**Leiden, The Netherlands, February 10, 2012.** Biotech company Pharming Group NV (“Pharming” or “the Company”) (NYSE Euronext: PHARM) today announced that it has started an open-label Phase II clinical study evaluating its recombinant human C1 inhibitor (rhC1INH; RUCONEST®) for the treatment of acute attacks of angioedema in pediatric patients with Hereditary Angioedema (HAE).

The protocol for the RUCONEST pediatric study has been agreed with the Pediatric Committee of the European Medicines Agency (EMA). The study will assess the pharmacokinetic, safety and efficacy profiles of RUCONEST at a dose of 50 U/kg in pediatric HAE patients in support of a pediatric indication for treatment of HAE attacks. Pharming expects to enroll approximately 20 pediatric patients by the end of the year, aged from 2 to 13 years. Further information on the study is available on [clinicaltrials.gov](http://clinicaltrials.gov).

Pharming also announced that the Company and its U.S. collaborator, Santarus, Inc., have discontinued a proof-of concept Phase II study in antibody-mediated rejection (AMR) after kidney transplantation. Recent improvements in clinical practice that significantly reduced the apparent incidence of AMR in renal transplant have decreased the need for therapeutic intervention, making patient recruitment for the clinical study difficult.

Pharming is continuing to evaluate rhC1INH for other potentially commercially attractive indications, such as ischemia reperfusion injury related indications and promising new platform derived projects like rhFVIII. Reperfusion injury is a complication arising from oxygen shortage due to an interruption of the blood supply (ischemia) resulting in tissue damage. This can occur in a transplanted organ, in the brain, in case of stroke, and in the heart, in case of myocardial infarction (heart attack).

Pharming has investigated the efficacy of rhC1INH in various pre-clinical reperfusion injury models with encouraging results and has recently (December 12, 2011) been granted a U.S. patent expiring in 2028, covering a method of preventing, reducing or treating an ischemia and/or reperfusion injury by administering certain recombinantly expressed C1 inhibitors (RUCONEST®/ RHUCIN®). This was Pharming’s first patent granted on ischemia/reperfusion injury in the U.S., and represents a significant milestone in the continuing development of the Company’s C1 inhibitor franchise in additional indications associated with the broad area of reperfusion injury.

### About RUCONEST (RHUCIN in non-European territories) 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 in Europe is approved under the name RUCONEST for treatment of acute angioedema attacks in patients with HAE. RHUCIN® is an investigational drug in the U.S., which 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® (RHUCIN® in non-European territories) 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. RHUCIN is partnered with Santarus, Inc. in North America where the drug is undergoing Phase III clinical development. The product is also being evaluated for additional indications. 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. Recently a new project, using the validated transgenic rabbit platform, aimed at the development of recombinant Factor VIII for the treatment of Haemophilia A was initiated. Additional information is available on the Pharming website, [www.pharming.com](http://www.pharming.com).

*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.*

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