# DNAGE INITIATES OBSERVATIONAL STUDY IN CHILDREN SUFFERING FROM COCKAYNE SYNDROME

**Leiden, The Netherlands, September 16, 2009.** Biotech company Pharming Group NV ("Pharming" or "the Company") (NYSE Euronext: PHARM) announced today that its wholly-owned subsidiary DNage has initiated an observational study in children suffering from Cockayne Syndrome (CS).

This study is designed to assess the natural (untreated) progression of the disease during a period of 6-12 months and will include detailed data collected from existing medical records as well prospective measurements and assessments. Data collected in the observational study will enable the Company to conduct one or more clinical studies to assess the efficacy of Prodarsan®, a product in development for the treatment of CS. Although the final design of these planned international efficacy studies is not yet completed and also dependent on the outcome of discussions with regulatory authorities, it is likely that in these studies Prodarsan® will be tested, amongst other parameters, for its effect on growth of paediatric patients compared to their growth when untreated.

DNage is developing Prodarsan® as a potential therapy for CS. The product is a combination of small molecules formulated formulated as an oral liquid and is believed to reduce the accumulation of DNA-damage, the underlying biochemical cause of Cockayne Syndrome. Prodarsan® has been successfully tested in animal models of CS and appeared to be safe and well-tolerated in a Phase I study in healthy human volunteers. Prodarsan® has an Orphan Drug Designation from the US Food and Drug Administration (FDA) who recently also approved an Investigational New Drug (IND) application allowing the Company to conduct a clinical trial in children suffering from CS.

The Principal Investigator of the observational study, Dr. Edward G. Neilan, Staff Physician at the Children's Hospital Boston/Harvard Medical School, commented: "The initiation of this observational study is an important milestone in the development of new treatment options for CS-patients and in particular for the development of Prodarsan®. For the first time, we will be collecting detailed measurements of the natural progression of this disease. This study will hopefully provide us with data needed to complete the design of a pivotal clinical study necessary to achieve registration of Prodarsan® as a therapeutic. While our center in Boston will serve as a central site in the current study, I look forward to collaborate with many colleagues in the US and abroad to make this study a success."

## **About Cockayne Syndrome**

CS is a premature ageing disease. Premature ageing diseases are a group of rare diseases caused by a genetic defect leading to deficient repair of DNA-damage. Patients suffering from these diseases develop multiple 'ageing-pathologies', normally associated with old age, early on in their lives. Generally, these patients have a strongly reduced quality of life and reduced life expectancy. CS is characterized (amongst other symptoms) by growth failure, mental retardation, hearing loss, a prematurely aged appearance (progeria) and premature death. The average lifespan of CS patients is 12.5 years and quality of life for these patients is seriously impaired. At present, there is neither a cure nor an effective therapy available for CS patients. Disease management consists of treating the symptoms as they arise and providing assistive devices.

#### **About DNage BV**

DNage is a wholly owned subsidiary of Pharming Group NV since its acquisition in 2006. DNage is developing products that interfere with the development of ageing-related pathologies. Its technology platform is based on the scientific work by the group of Dr. Hoeijmakers, at the Erasmus Medical Centre in Rotterdam, who established a link between insufficient DNA-repair (accumulation of DNA-damage) and the development of ageing-related diseases. DNage has active programs in the field of neurodegeneration, bone disease and other ageing-related pathologies. Additional information is available on the Pharming website, <a href="http://www.pharming.com">http://www.pharming.com</a> and on <a href="http://www.dnage.nl">http://www.dnage.nl</a>

### **About Pharming Group NV**

Pharming Group NV is developing innovative products for the treatment of genetic disorders, ageing diseases, specialty products for surgical indications, intermediates for various applications and nutritional products. Pharming has two products in late stage development - Rhucin® for Hereditary Angioedema and human Lactoferrin for use in food products and one product in early stage clinical development - Prodarsan® for Cockayne Syndrome. The advanced technologies of the Company include innovative platforms for the production of protein therapeutics, technology and processes for the purification and formulation of these products, as well as technology in the field of DNA repair (via DNage). Additional information is available on the Pharming website, 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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