

## FDA APPROVES INVESTIGATIONAL NEW DRUG APPLICATION FOR PRODARSAN® IN COCKAYNE SYNDROME PATIENTS

**Leiden, The Netherlands, August 14, 2009.** Biotech company Pharming Group NV (“Pharming” or “the Company”) (NYSE Euronext: PHARM) announced today that its wholly-owned subsidiary, DNage, has had its Investigational New Drug (IND) application for Prodarsan® accepted by the US Food and Drug Administration (FDA), allowing DNage to initiate a clinical trial in children suffering from Cockayne Syndrome (CS).

In this short clinical study, which is expected to be conducted in the fourth quarter of 2009, the pharmacokinetics (PK) of single and multiple escalating doses of Prodarsan® will be evaluated in a small number of paediatric patients with CS. The individual components of Prodarsan® are already known to the FDA and during this study data will be collected to make a bridge to existing data on the individual components of Prodarsan®. This will facilitate the future registration of Prodarsan®. In addition to data collected in previous studies, the safety and tolerability of the product will also be investigated in this study. The Principal Investigator is Dr. Edward G. Neilan, Staff Physician at the Children's Hospital Boston/Harvard Medical School, a world-renowned clinician and researcher in the field of Cockayne Syndrome.

Prodarsan® is a product in development to delay the progression of CS, 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-related 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. Prodarsan® is a combination of small molecules formulated as a liquid for oral use and is believed to reduce the accumulation of DNA-damage, the underlying biochemical cause of CS. There is currently no effective therapy available for this disease.

In late 2008, a Phase I study of Prodarsan® in healthy human volunteers was successfully completed. In this study, single and multiple escalating doses were administered to healthy volunteers to evaluate the PK, safety and tolerability of Prodarsan® in adults. Prodarsan® appeared to be safe and well tolerated in dosages that had earlier shown to yield significant beneficial effects in animal models of CS. Since Prodarsan® will be used as an oral, liquid formulation the effects of food intake on absorption and elimination of the product were also investigated. Based on these data, combined with data generated in the preclinical program, an oral dosing scheme has been established that will be used in the upcoming studies in paediatric CS patients.

After successful completion of the planned clinical study and the ongoing toxicology testing, DNage expects to perform another clinical trial to evaluate the efficacy of Prodarsan® in alleviating symptoms of CS. The final design and set-up of this planned international study will be completed after discussions with regulatory agencies and evaluating the results of the currently planned short clinical study.

Dr. Rein Strijker, General Manager of DNage and Chief Commercial Officer at Pharming, commented: “Prodarsan®, as the first product being developed with DNage technology, is testimony to the great potential of this approach in treating diseases related to ageing in humans. The positive data with Prodarsan® in preclinical and the earlier completed Phase I studies are encouraging for its further development. We look forward to initiating studies in CS patients and will also continue to develop a number of other product

candidates for the treatment of other premature ageing diseases as well as ageing diseases in the elderly population. With the continued increase in average life-expectancy of humans in many parts of the world, there is a great medical need for new products in the field of diseases associated with old age. We believe that our innovative DNage technology can contribute to develop new approaches in this area.”

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## **Background on Investigational New Drug (IND) Application**

An Investigational New Drug application is a request for authorization from the US Food and Drug Administration to administer an investigational drug or biological product to humans consistent with an approved protocol. Such authorization must be secured prior to administration of any new drug or biological product. The IND application contains information on the product's preclinical and clinical results, manufacturing data, and detailed clinical protocols for proposed clinical studies. An IND approval confirms that the FDA agrees that the product can be tested in humans to collect information pertinent to the product's safety and efficacy. The IND-application for Prodarsan® was filed by Clinquest, DNage's agent in the USA.

## **Background on Cockayne Syndrome**

Cockayne Syndrome is a rare disease and is one of a collection of premature ageing disorders. CS is a genetic disorder that is (amongst other symptoms) characterized by growth failure, mental retardation, hearing loss, a premature 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. In April 2009, DNage received an Orphan Drug designation from the FDA for Prodarsan®. The Orphan Drug designation provides for an accelerated review process, tax benefits, exemption from user fees and a seven-year period of market exclusivity in the US after product approval.

## **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. In addition to the DNage programs Pharming is developing innovative products for the treatment of genetic disorders, 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. Additional information is available on the Pharming website, <http://www.pharming.com> and on <http://www.dnage.nl>.

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