



## PRESS RELEASE

### **Prosensa's RNA based therapeutic PRO051 shows favourable results in the first systemic study in patients with Duchenne muscular dystrophy**

*Preparations for a Phase III study are underway*

**Leiden, September 14, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics announces positive results from its phase I/IIa clinical study on its lead product candidate: the systemic delivery of PRO051 in patients with Duchenne muscular dystrophy (DMD) was well tolerated and induced novel expression of dystrophin. The data of the clinical study were presented at the 14<sup>th</sup> Annual International Congress of the World Muscle Society (WMS) in Geneva, Switzerland by Dr Nathalie M. Goemans, MD, from the Department of Pediatric Neurology, University of Leuven, Belgium.**

The completed phase I/IIa open label study evaluated the systemic delivery of PRO051 in twelve DMD patients receiving weekly subcutaneous injections over a period of five weeks. Four dosing cohorts were applied (0.5 mg/kg, 2 mg/kg, 4 mg/kg, 6 mg/kg), comprising three patients each. The effect of PRO051 was assessed at the RNA level, to demonstrate specific exon 51 skipping, and at the protein level, to demonstrate novel dystrophin expression.

Muscle biopsies were taken before and two weeks after the last administration for patients in cohort 1, and at two and seven weeks after the last administration for all other patients. Adverse events were recorded and safety assessments (laboratory analysis and ECG) performed at regular intervals. All patients in this study entered a long term extended treatment phase.

Subcutaneous administration of PRO051 resulted in specific exon 51 skipping in cohorts 2, 3 and 4, and induced dystrophin expression in a dose related manner in all cohorts. The treatment was well tolerated by all patients and none discontinued their participation in the study. A review of the safety data revealed no clinically significant changes in laboratory values and ECGs. Antibodies against dystrophin were not detected in any of the patients.

The study was performed in close collaboration with the Universities of Leuven, Belgium and Gothenburg, Sweden. Dr. Goemans commented: "The data from this first Phase I/IIa study evaluating the systemic delivery of PRO051 are very promising. The subcutaneous administration was straightforward and well tolerated."

Commenting on the study, key investigator Dr. Tulinius from The Queen Silvia Children's Hospital in Gothenburg, Sweden, said: "Currently available therapies for DMD alleviate symptoms, but they do not address the underlying cause of this serious disease. The completion of this first systemic delivery PRO051 study is therefore an important next step in the development of new treatment options for patients with DMD."



“These study results are an important milestone for Prosensa. They move our development program into a next phase and strengthen our position as a frontrunner in the field of RNA based therapeutics, in particular exon skipping. We are now preparing a double blind placebo controlled phase III study. This confirms our commitment to provide patients with new innovative treatments that will help them to live a better life” said Hans Schikan, CEO of Prosensa.

### **About Prosensa**

Prosensa is a highly innovative Dutch biopharmaceutical company focused on the discovery, development and commercialization of nucleic acid based therapeutics correcting gene expression in diseases with large unmet medical needs, in particular neuromuscular disorders. Prosensa is focused on developing a treatment for DMD (Duchenne Muscular Dystrophy). Prosensa’s lead compound PRO051 is currently in advanced phase I/II clinical trials and the company anticipates starting a phase III trial in 2010. The company recently concluded a successful series B financing round of EUR 18 million with a consortium of esteemed investors. For more information about Prosensa, please visit [www.prosensa.eu](http://www.prosensa.eu).

### **About DMD and exon skipping**

Duchenne muscular dystrophy is a severely debilitating childhood neuromuscular disease that affects 1 in 3,500 newborn boys. The young patients suffer from progressive loss of muscle strength due to the absence of the dystrophin protein, making them wheelchair bound before the age of 12 and most die in early adulthood due to respiratory and cardiac failure. Today, there is no treatment to prevent the eventual fatal outcome. The disease is caused by mutations in the DMD gene, resulting in the absence of the dystrophin, which is crucial for the integrity of muscle fiber membranes.

RNA-based therapy applying antisense oligonucleotides to induce specific exon skipping, is currently one of the most promising new potential therapies for DMD. Antisense oligonucleotides have the capacity to correct the mutated reading frame of DMD transcripts and to introduce the synthesis of a novel, partially to largely functional, dystrophin protein. Different mutations in the gene require different oligonucleotide drugs. PRO051, the first of its kind, may be therapeutic for approximately 13% of all DMD patients.

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