



PRESS RELEASE

**Prosensa to Present Data at the World Muscle Society Congress**

**Leiden, September 8, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics announces two scheduled presentations of data on its lead product candidate, PRO-051, at the 14<sup>th</sup> International Congress of the World Muscle Society, September 9-12, 2009 in Geneva, Switzerland.**

Dr. Judith van Deutekom, PhD, Senior Director Discovery at Prosensa will present an overview of the pre-clinical development of Prosensa's product portfolio for the treatment of Duchenne muscular dystrophy, including PRO-051.

Prof. Dr. Nathalie M. Goemans, MD, from the Center of Metabolic Diseases, University Hospital Gasthuisberg, Leuven, Belgium, will present the phase I/IIa results of Prosensa's lead compound PRO-051 in patients with Duchenne muscular dystrophy.

Presentations:

**Title:**

"Making Sense of Duchenne – The Role of Preclinical Development"  
Judith van Deutekom

**Date and time of the session:**

At the Special Industry Forum: Emerging Therapies for Neuromuscular Diseases on  
Thursday, September 10 from 19:10 – 19:30 in the auditorium R080

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**Title:**

"A phase I/II study on antisense compound PRO-051 in patients with Duchenne  
muscular dystrophy"

N.M. Goemans; G. Buyse; M. Tulinius; J.J.G. Verschuuren; S.J. de Kimpe; J.C.T. van  
Deutekom

**Date and time of the session:**

Saturday, September 12 from 14:30 – 15:00 in the auditorium R380

**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 PRO-051 is currently in advanced phase II clinical trials and the company anticipates starting a phase III trial early next year. Prosensa is a private company backed by 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 protein dystrophin, 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 protein, which is crucial for the integrity of muscle fiber membranes.

RNA-based therapeutics, specifically antisense oligonucleotides inducing exon skipping, are currently amongst the most promising therapy for DMD. More specifically, antisense oligonucleotides have the capacity to skip an exon and thereby correct the reading frame of DMD transcripts aiming at the synthesis of a largely functional dystrophin protein. Different mutations in the gene require different oligonucleotide drugs. The PRO-051, the first of its kind, will be suitable for approximately 13% of all DMD patients.

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