

**PRESS RELEASE, May 5<sup>th</sup>, 2008**

**Prosensa announces the start of an international multi-center phase I/II clinical study with 'smart drug' PRO051 in patients with Duchenne Muscular Dystrophy**

On May 5<sup>th</sup>, 2008, Prosensa announces that it has started a phase I/II study to explore the effect, safety and tolerability of systemic injections of PRO051 in Duchenne Muscular Dystrophy (DMD) patients. This trial is performed in collaboration with UZ Leuven (Belgium), the Queen Silvia Children's hospital (Sweden) and the LUMC (the Netherlands). The UZ Leuven and the Queen Silvia Children's hospital have already started to enroll patients. In this study, an important parameter will be the presence of dystrophin in muscle biopsies, the protein missing in patients with Duchenne Muscular Dystrophy (DMD). This clinical trial in patients with DMD uses an antisense oligoribonucleotide, a 'smart drug' removing an unwanted segment of the faulty DMD gene product, and represents a novel approach to combat genetic diseases like DMD.

Dr. Nathalie Goemans, the coordinating and principle investigator in Leuven says: "I am excited that, based on the encouraging proof of concept data from the previous clinical study, we can now proceed with this next important step in the investigations that are required to determine whether this highly promising approach can be developed into an effective and safe treatment for patients with this devastating disease. "

"We are proud that quickly after completion and publication of the first trial we are able to conduct this study. We expect to provide further information on the developments later this year. In this study, we extend our recent success proving the concept of local dystrophin production to a study with systemic application to achieve widespread dystrophin expression in muscles. ", says Gerard Platenburg, Prosensa's CEO.

**ABOUT DMD AND EXON SKIPPING**

Duchenne muscular dystrophy is a severely debilitating childhood neuromuscular disease that affects 1 in 3500 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 the most promising therapy for Duchenne Muscular Dystrophy. More specifically, antisense oligonucleotides have the capacity to skip an exon and thereby correct the reading frame of *DMD* transcripts resulting in the synthesis of a largely functional dystrophin protein. Different mutations in the gene require different oligonucleotide drugs. The PRO051, the first of its kind, will be suitable for 15% of all DMD patients, because it can treat a cluster of mutations.

**ABOUT PROSENSA**

Prosensa is a Dutch biopharmaceutical company dedicated to the development of RNA-based therapeutics targeting diseases with unmet medical needs, in particular neuromuscular disorders. Prosensa's drug development is based upon a unique and

proprietary technology platform involving 'exon skipping', enabling correction of mutated RNA. This ability to modulate genes selectively through RNA-based therapeutics could provide a new way to treat a wide range of human diseases. The company has, together with its partner LUMC, a leadership position in fundamental patents, technology, and know-how relating to RNA-based approaches and exon skipping. Prosensa's lead compounds for DMD are currently in clinical phase I/II development and the company has received orphan drug designation both in Europe and the US. Prosensa is elected the European Venture Company of 2007. For more information on Prosensa, please visit [www.prosensa.nl](http://www.prosensa.nl).

#### **ABOUT UZ LEUVEN**

The 1 894 beds at the University Hospitals of Leuven (UZ Leuven) make this the largest hospital in the country. UZ Leuven sees its task as caring for patients, conducting research and providing training at academic level. The Neuromuscular Reference Center for Children serves as a centralized clinical center for diagnosis and management of neuromuscular patients, and provides required facilities for conducting and participating in clinical trials and has a longstanding experience in conducting clinical trials and translational research in Duchenne Muscular Dystrophy.

#### **ABOUT THE QUEEN SILVIA CHILDREN'S HOSPITAL IN GÖTEBORG**

The Queen Silvia Children's Hospital in Göteborg is one of the leading neuromuscular centers in Sweden and plays an active role in research, and offers diagnostic work-up and yearly follow-up programs for children with all kinds of neuromuscular disorders.

#### **ABOUT LUMC**

Leiden University Medical Centre (LUMC) aims to play a leading role, nationally and internationally, in the further improvement of health care quality. LUMC's key tasks are research, patient care, and academic and post-academic medical education. It performs 11,500 daytime treatments and 19,000 hospital admissions yearly. It has 800 beds and employs 8700 people. For more information see [www.lumc.nl](http://www.lumc.nl).

For more information on Prosensa, please contact:  
Gerard Platenburg, CEO, [g.platenburg@prosensa.nl](mailto:g.platenburg@prosensa.nl)

For more information on the clinical study, please contact:  
Ad Sitsen, CMO, [a.sitsen@prosensa.nl](mailto:a.sitsen@prosensa.nl)