

Leiden University Medical Center and Prosensa B.V. Announce New England Journal of Medicine Publication of First Successful Clinical Study with RNA-based Therapeutic PRO051 in Duchenne Muscular Dystrophy

Novel RNA-based therapeutic PRO051 to enter Phase I/II clinical trials in DMD

Leiden, the Netherlands, December 27, 2007. Leiden University Medical Center (LUMC) and Biotech Company Prosensa announced today in the *New England Journal of Medicine* (van Deutekom et al.) positive results from the first ever clinical study with RNA-based therapeutic PRO051 in four patients with Duchenne Muscular Dystrophy (DMD), in which PRO051 has restored dystrophin expression in the treated muscle fibers of all four patients with Duchenne Muscular Dystrophy (DMD) tested.

In this study, DMD patients between 10 and 13 years of age received a single injection of PRO051 (a 2'-O-methyl antisense oligonucleotide) in a small area of a muscle in the lower leg. In a biopsy taken four weeks later, novel dystrophin expression was observed in the vast majority of muscle fibers with protein levels that are expected to be clinically relevant. This pilot study is the first ever with an RNA-based therapeutic agent for DMD and is an important step towards treatment of this devastating disease, for which as yet no therapy is available. Following this first clinical proof-of-mechanism, Prosensa has started the preparations for a phase I/II clinical study to explore the effects and safety of PRO051 after repeated systemic injections.

Gerard Platenburg, Prosensa's CEO, further emphasized: "This first clinical study demonstrates that PRO051, an RNA-Therapeutic based on our proprietary exon skipping technology, was able to correct this genetic error in locally injected muscle tissue of Duchenne Muscular Dystrophy patients. We are excited to have demonstrated clinical proof-of-mechanism and will be progressing PRO051 into systemic Phase I/II trials in order to assess the broader benefits available to patients. RNA-based therapeutics hold great promise as an approach to create a whole new class of innovative medicines and we are well positioned to exploit this opportunity."

Professor Gertjan van Ommen, together with his colleagues Dr. Judith van Deutekom and Dr. Jan Verschuuren responsible for the research at the LUMC commented: "After eight years of preparatory work we were greatly rewarded by this wide-spread expression of dystrophin throughout the muscle biopsies" and "The robust expression in all four patients with different mutations after a single injection points to a solid effect. The results form an excellent starting point for systemic treatment trials".

Professor Eric Hoffman, one of the pioneers who identified dystrophin as the missing protein in DMD in 1987, remarked in a commenting editorial that "the paper by Van Deutekom et al. might herald the dawn of personalized molecular medicine".

Elizabeth Vroom, President of the United Parent Projects Muscular Dystrophy emphasized the enthusiasm amongst the patient community: "Duchenne parents all over the world are incredibly excited about the extremely promising results of the first human exon-skipping trial, which took place in the Netherlands. We greatly appreciate all the work the research team has put into this trial and are desperately hoping for positive results in the next stage also."

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 wheelchairbound 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 13% of all DMD patients, because it can treat a cluster of mutations.

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.

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 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. For more information on Prosensa, please visit www.prosensa.nl.

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