Leiden, August 17, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics announces that it has received notice of allowance for a US patent protecting the composition of matter of its lead compound PRO-051.

The US patent entitled “Modulation of exon recognition in pre-mRNA by interfering with the secondary RNA structure” describes and claims a group of oligonucleotides that can be used for targeted modulation of pre-mRNA, a method to restore genetic defects in the dystrophin gene of patients with Duchenne Muscular Dystrophy (DMD). The claimed oligonucleotides form the basis of PRO-051, Prosensa’s lead compound for the treatment of DMD which is in advanced phase II development. The company also enjoys protection for PRO-051 in many other regions, amongst which Europe. Recently, a Request for Opinion on the validity of the UK patent originating from a granted European patent was filed by a third party at the UK patent office. The UK patent office confirmed the validity of this UK patent, thereby confirming the decision of the European Patent office to grant said European patent.

This US patent is part of a portfolio of patents that was exclusively licensed from the Leiden University Medical Center (LUMC, Leiden, the Netherlands). The first inventor and current Director Discovery at Prosensa, Dr. Judith van Deutekom, was former head of the DMD Genetic Therapy group at LUMC and one of the pioneers in the development of genetic therapies for muscular dystrophies.

“This patent family sits at the core of our exon skipping technology platform and the notice of allowance of this patent significantly strengthens our proprietary position in this field. Moreover, the speed in which the US Patent Office has granted our patent application points to the clear and undisputed novelty an inventiveness of our products and is a further example of the pioneering nature of our technology,” comments Dr. van Deutekom. “We will continue to broadly file patent applications to build a strong intellectual property portfolio around our proprietary technology and to further strengthen our leadership position in this emerging field of RNA-based therapeutics.”

“I’m extremely pleased with the notice of allowance for this US patent as it provides us with a broad protection for our lead therapeutic candidate PRO-051. It confirms our leadership position in the field and we will continue to diligently develop the product in order to hopefully bring a solution to patients in a timely manner” said Hans Schikan, Chief Executive Officer 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 PRO-051 is currently in advanced phase II clinical trials and the company anticipates starting a phase III trial before the end of 2009. 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.

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 8,700 people. For more information see www.lumc.nl.

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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