



PRESS RELEASE

Prosensa and LUMC strengthen strategic alliance through broadened RNA modulation license agreement

Leiden, September 30, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA therapeutics and Leiden University Medical Center announce that they have extended their license agreement on RNA modulation.

In 2003, Prosensa obtained an exclusive world-wide license from Leiden University Medical Center (LUMC, Leiden, the Netherlands) for the application of its proprietary RNA modulation technology in the field of neuromuscular diseases such as muscular dystrophies. This technology was developed at the Human Genetics department of Prof. Dr. Gert-Jan van Ommen. The recent extension of the license agreement gives Prosensa the rights to apply the RNA modulation technology for the development of treatments for indications outside the field of neuromuscular disorders.

“Following the proof-of-concept that we obtained with this exciting therapeutic approach for the treatment of Duchenne muscular dystrophy, we see a clear opportunity in applying this powerful technology platform in other indications and building a broad product portfolio for Prosensa. We are very pleased with the collaboration with LUMC and privileged to be able to work closely with the strong scientific team under the leadership of professor van Ommen” says Hans Schikan, CEO of Prosensa.

Van Ommen, head of the LUMC Human Genetics Department and a pioneer in RNA modulation research comments: “Prosensa has been an ideal partner for us to translate the results of our research into solutions to treat patients, as demonstrated by the tremendous progress that has been made in the development of novel and promising treatments for Duchenne muscular dystrophy. Therefore it was an obvious decision for us to grant Prosensa the rights to use our RNA modulation platform to develop treatments for other debilitating and life-threatening diseases.”

“We are very happy with this extended agreement. LUMC has achieved a major breakthrough with the RNA modulation platform and has been able to secure a strong patent position in this field. This broadened license will allow us to further build our internal pipeline with novel applications. It will also further establish Prosensa as the partner of choice for other companies looking to enter this exciting new area” comments Luc Dochez, VP Business Development 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). Following a successful proof of principle trial in 2007 Prosensa's lead compound PRO051 is now also showing great promise in an advanced phase I/II clinical trial, and the company anticipates starting a phase III trial early next year. For more information about Prosensa, please visit www.prosensa.eu.



About LUMC

Leiden University Medical Centre (LUMC) is a center for medical innovation, committed to the advancement of health care through research and innovation. Its focus is on translational research to accelerate the trajectory from the laboratory findings to clinical application and into the market place. As academic medical center it provides patient care and medical education. LUMC performs 12,000 daytime treatments and 20,000 hospital admissions yearly, has 800 beds and employs 7,500 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 often 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 therapies 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. PRO051, the first of its kind, will be suitable for approximately 13% of all DMD patients.

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