PRESS RELEASE

Prosensa and TREAT-NMD enter into strategic collaboration for PRO-051 clinical trial planning

Leiden, August 31, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics, announces the successful completion of a feasibility enquiry using the TREAT-NMD Global Database for DMD and the TREAT-NMD Care and Trial Sites Registry for the planning of the phase II/III study for its lead compound PRO-051.

Prosensa recently completed a phase I/II clinical trial for PRO-051, its lead compound for the treatment of Duchenne Muscular Dystrophy (DMD), and the company anticipates starting a phase II/III clinical study early next year. PRO-051 is directed to a specific mutation in the dystrophin gene that occurs in approximately 13% of the DMD patient population. In order to set up the pivotal study, patients with a genetic mutation amenable to exon skipping by PRO-051 need to be selected.

TREAT-NMD has developed a global database that contains precise genetic and clinical information from patients with DMD, including age, ambulation status and medication use. Using the TREAT-NMD Global Database for DMD, Prosensa has identified around 300 patients from 21 countries who meet the inclusion criteria for the upcoming trial. The TREAT-NMD database holds up-to-date information about all these patients that will allow them to be contacted for trial recruitment purposes through the national registries. These patients were matched to 50 potential trial sites and selected patients and sites will be contacted for participation in the upcoming clinical trial.

“Patient recruitment for clinical studies is a very expensive and time-consuming process, in particular for rare diseases such as DMD” comments Dr. Giles Campion, CMO and VP R&D of Prosensa. “Therefore, this successful collaboration with TREAT-NMD is extremely valuable for us as it facilitates the acceleration of the recruitment process and hopefully allows us to bring this treatment faster to patients.”

“We are delighted that our global patient database is providing such useful results for pharmaceutical companies like Prosensa,” said Professor Hanns Lochmüller, leader of the TREAT-NMD patient registries initiative. “Our approach is unique because the data we are giving companies isn’t just generic statistical information. It is up-to-date information about real patients who can be recruited into clinical trials through the registry or contacted when a treatment is available. At the same time, it’s safe for patients, since we don’t give out identifiable information to companies but act as a trusted intermediary and all our registries have full ethical approval and comply with data protection laws.”

Prosensa and TREAT-NMD will both be attending and presenting at the World Muscle Society Conference in Geneva from September 9th till 12th.

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). For more information about Prosensa, please visit www.prosensa.eu.

About TREAT-NMD
TREAT-NMD is a Network of Excellence facilitating collaborative research in neuromuscular disease that aims to create the infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. Since the network was launched in January 2007 it has built up the tools that industry and researchers need to bring promising new therapies more quickly from the lab to the clinic. One of the key TREAT-NMD infrastructures built up in the last two years is a global patient registry for DMD and SMA comprising more than 30 national patient registries worldwide. The DMD registries now hold more than 9,000 individual patient entries with standardized items and patient consent, facilitating and accelerating clinical research and trials while giving patients improved access to relevant information on standards of diagnosis and care. These registries have been set up in collaboration with clinicians and patient organizations across the world and contain the key information needed to establish whether a particular patient might be eligible for a trial, together with the means of contacting them. Registries for other conditions are also in preparation. For more information, please visit www.treat-nmd.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, often making them wheelchair bound before the age of 12. Most patients 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. PRO-051, the first of its kind, will be suitable for approximately 13% of all DMD patients.

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