



PRESS RELEASE

Prosensa and GlaxoSmithKline form alliance to fight Duchenne Muscular Dystrophy

Leiden and London, October 13, 2009 – Prosensa, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics, and GlaxoSmithKline (LSE: GSK) announce that they have entered into an exclusive worldwide collaboration for the development and commercialization of RNA based therapeutics for Duchenne Muscular Dystrophy (DMD). DMD is a severely debilitating childhood neuromuscular disease that affects one in 3,500 newborn boys. Currently, there is no treatment to prevent the eventual fatal outcome.

The alliance was established under GSK's Centre of Excellence for External Drug Discovery (ceedd) which seeks to collaborate with companies at the leading edge of highly innovative and transformative science. The scope of the alliance includes four RNA-based products intended to treat specific, but different, subpopulations of patients suffering from DMD.

Under the terms of the agreement, GSK will obtain an exclusive worldwide license to develop and commercialize Prosensa's lead compound, PRO051, intended to treat DMD by skipping exon 51 of the dystrophin gene. Mutations in the dystrophin gene result in the absence of normal dystrophin protein, which is necessary for proper muscle cell function. GSK's Neurosciences Medicines Development Centre will continue to progress the further development of PRO051 in collaboration with Prosensa. Both parties have begun preparations for a Phase III study which is intended to start in early 2010. GSK will fund all costs associated with the further clinical development of PRO051. In addition, GSK has exclusive options to license three more RNA-based compounds targeting additional DMD exons. One such option includes Prosensa's second lead compound, PRO044, which targets the skipping of exon 44 and for which Prosensa expects to initiate a Phase I/II study before the end of 2009. In this case, GSK's option rights will be triggered by a successful completion of this study.

The financial terms include a GBP 16 million (USD 25 million) upfront payment. Furthermore, Prosensa is eligible to receive up to GBP 412 million (USD 655 million) in milestones payments if all four compounds are successfully developed and is also entitled to double-digit royalties on product sales. Prosensa will retain commercial participatory rights, and has an option to expand its commercial rights, in certain European countries on products arising under the collaboration.

Hans Schikan, CEO of Prosensa said: "We are delighted by GSK's commitment to develop and commercialize our promising lead compound, PRO051. This alliance will not only speed-up the further development of PRO051, but will also accelerate the progress of our complementary DMD therapeutics, allowing us to reach a broader patient population. Our joint commitment to serve Duchenne patients provides a solid basis to achieve our goal to improve the lives of these boys and their families."

"We are always looking for inspiring science to take forward and are incredibly excited at the prospect of working with Prosensa to bring a vital new treatment to patients with





DMD," said Dr. Christoph Westphal, CEO of Sirtris, a GSK company, and SVP and Head of ceedd, GSK.

"PRO051 has generated a great deal of interest from the pharmaceutical industry. This strategic alliance with a premier pharmaceutical company is an acknowledgement of our business strategy and of the strength of our technology platform, which was jointly developed with Leiden University Medical Center. It is a transformative event for Prosensa that will enable us to accelerate the development of our pipeline of RNA modulating therapeutics and to broaden our development scope into other therapeutic areas with an unmet medical need," said Luc Dochez, Vice President Business Development at Prosensa.

About DMD and exon skipping

DMD is a severely debilitating childhood neuromuscular disease that affects one in 3,500 newborn boys. These 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, 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 dystrophin 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.

About GlaxoSmithKline

GSK is enhancing the way it discovers and develops new medicines by increasing external alliances. The Centre of Excellence for External Drug Discovery (ceedd) seeks out alliances with world class biotech companies with cutting edge technologies and scientific platforms that can support drug discovery and development. For information about the ceedd, visit the group's website at www.ceedd.com.

GlaxoSmithKline - one of the world's leading research-based pharmaceutical and healthcare companies - is committed to improving the quality of human life by enabling people to do more, feel better and live longer. For further information please visit www.gsk.com.

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. Prosensa's lead compound PRO051 has shown great promise in an advanced phase I/II clinical trial, and will enter a phase III trial early next year. For more information about Prosensa, please visit www.prosensa.eu.

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