Prosensa Announces Progress on Exon Skipping Compounds for the Treatment of Duchenne Muscular Dystrophy

£10m in milestone payments received from GlaxoSmithKline

Leiden, The Netherlands, 23 October 2012 – Prosensa, the Dutch biopharmaceutical company focusing on RNA-modulating therapeutics for rare diseases with high unmet need, has selected clinical candidates for two more compounds for the treatment of Duchenne muscular dystrophy (DMD) and has been granted orphan drug designation for two additional DMD compounds in its pipeline.

Prosensa identified suitable oligonucleotide candidates for PRO052 and PRO055, designed for the skipping of exons 52 and 55 of the dystrophin gene. PRO052 and PRO055 are currently in pre-clinical development and will be moved to clinical trials as soon as possible.

Prosensa has also received orphan drug designation by the European Commission for PRO045 and PRO053. These compounds are designed to skip exons 45 and 53 of the dystrophin gene and are expected to enter clinical development within the next 6 months. In parallel, Prosensa and GSK have initiated a large global natural history study in order to generate important data on the progress of DMD that will help to facilitate the development pathways for these compounds.

Together with drisapersen (PRO051/GSK2402968) and PRO044, already in clinical development, the Prosensa portfolio of six DMD compounds has the potential to treat more than 40% of all DMD patients.

Hans Schikan, CEO of Prosensa, commented: “Achieving orphan drug status for PRO045 and PRO053 is an important milestone that we believe will allow us to accelerate development of these compounds and initiate clinical trials within the next half year. While GSK has an option to license one compound, PRO045 or PRO053, the other compound will be developed and eventually commercialized by Prosensa in our ambition to become a fully integrated biopharmaceutical player in rare diseases. Together with PRO052 and PRO055, where the same principle applies, Prosensa will be in a position to target rare mutations in specific DMD subpopulations and may provide treatment to more patients with this debilitating disease.”

Prosensa has the most advanced portfolio of drug candidates for the treatment of DMD, and has also preclinical compounds for Myotonic Dystrophy and Huntington’s Disease. Prosensa’s DMD compounds are based on its proprietary exon-skipping technology that uses antisense oligonucleotides to restore expression of a functional dystrophin protein and to provide potential treatment for patients affected by this progressively debilitating neuromuscular disease.

—ENDS—
Notes to editors:

About DMD
Duchenne muscular dystrophy (DMD) is a severely debilitating childhood neuromuscular disease that affects 1 in 3,500 live male births. This rare disease is caused by mutations in the dystrophin gene, resulting in the absence or defect of the dystrophin protein. As a result, patients suffer from progressive loss of muscle strength, often rendering them wheelchair-bound before the age of 12. Respiratory and cardiac muscle can also be affected by the disease and most patients die in early adulthood due to respiratory and cardiac failure.

About exon skipping
The dystrophin gene is the largest gene in the body, consisting of 79 exons. Exons are small sequences of genetic code which, via an intermediate step involving RNA, lead to the assembly of sections of protein. In DMD, when certain exons are mutated/deleted, the RNA cannot read past the fault. This prevents the remainder of the exons from being read, resulting in a non-functional dystrophin protein and the severe symptoms of DMD. RNA-based therapeutics, specifically antisense oligonucleotides inducing exon skipping, are currently in development for DMD. These antisense oligonucleotides skip an exon next to a defective exon and thereby correct the reading frame, enabling the production of a novel, functional dystrophin protein. Prosensa’s exon skipping technology originated in Leiden University Medical Center.

About Prosensa
Prosensa is an innovative Dutch biopharmaceutical company focused on the discovery, development and commercialization of RNA-modulating therapeutics correcting gene expression in diseases with significant unmet need, in particular neuromuscular disorders. Prosensa’s current focus is on developing treatments for Duchenne muscular dystrophy (DMD), Myotonic Dystrophy and Huntington’s disease. In 2009 Prosensa entered into a strategic alliance for part of its DMD exon skipping program with GlaxoSmithKline. Prosensa’s lead compound (drisapersen), being developed by GSK, is currently in phase III clinical trials. Prosensa is a privately held biopharmaceutical company, backed by a consortium of Abingworth, Gimv, Idinvest Partners, Life Sciences Partners, MedSciences Capital and New Enterprise Associates. For more information, please visit www.prosensa.com.

Prosensa won the 2012 Emerging Star Award at the European Mediscience Awards and was listed as a Fierce 15 Biotech Company.

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