Prosensa and GlaxoSmithKline Initiate Development of Four Additional Products under Existing Alliance in Duchenne Muscular Dystrophy; Broadened program marks key inflexion in Prosensa's progress to a fully integrated specialty-pharma company

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Body

Prosensa, the Dutch biopharmaceutical company focusing on RNA modulating therapeutics, announces the initiation of two further programmes under its existing alliance with GSK covering novel RNA-based treatments for Duchenne Muscular Dystrophy (DMD). The initiation of these additional programs under the terms of the existing alliance agreement is a validation of both the potential of Prosensa's "exon skipping" platform and the ongoing relationship.

The two new programs are included under the existing alliance agreement between both parties and will address the development of four compounds which target different subpopulations of patients suffering from DMD. To access these new programs, GSK has made two initiation payments to Prosensa and Prosensa becomes eligible for further pre-option milestone payments based on research progress.

Within these new programs, Prosensa and GSK will focus on the skipping of four exons (i.e. exon 45, 52, 53, and 55), in addition to their existing programs which target skipping of exon 51 and 44 (PRO051/GSK2402968 and PRO044). The initiation of these programs confirms the joint commitment of both companies to find treatments for DMD.

Under the terms of the collaboration, GSK has an option to select two of these additional four compounds for later-stage development and commercialization. Prosensa will retain certain limited European commercialization rights alongside GSK for the two compounds selected by GSK. For the two compounds not selected by GSK, Prosensa will retain full commercialization rights.

"We are very pleased with this news. Prosensa and GSK's commitment to progress further developments of additional products that can provide for a solution in DMD is encouraging and welcomed by all of us" said Elizabeth Vroom, Chair of the United Parent Project Muscular Dystrophy, which unites different parent project organizations set up by parents of children with DMD in many countries all over the world.

"The expansion of our DMD portfolio with four additional compounds is a great step forward in our efforts to develop safe and effective treatments for this severely debilitating disease and to help as many patients as possible," said Hans Schikan, Chief Executive Officer of Prosensa, adding, "Thanks to the successful progress in our existing program with GSK, we are now able to extend our productive collaboration with these four further compounds. With Prosensa retaining full commercialization rights to two of these exon skipping compounds and any other products which are not part of the alliance with GSK, this set-up enables us to develop into a fully integrated specialty-pharma company."

Notes to editors:

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 large unmet medical needs, in particular neuromuscular disorders. Prosensa's focus is on developing a treatment for DMD. In 2009 Prosensa entered into a strategic alliance for part of its DMD exon skipping program with GlaxoSmithKline in a licensing deal that could yield up to GBP 437 million plus double-digit royalty payments.

Prosensa is a privately held biopharmaceutical company, backed by a consortium of highly regarded investors such as Abingworth, AGF Private Equity, GIMV, LSP and MedSciences Capital.For more information about Prosensa, please visit www.prosensa.com .

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 (for a graphical explanation of exon skipping, click here). Different mutations in the gene require different oligonucleotide drugs.

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