



Prosensa announces commencement of re-dosing of drisapersen in North America in patients with Duchenne muscular dystrophy

Leiden, The Netherlands, Sept. 17, 2014 (GLOBE NEWSWIRE) -- Prosensa Holding N.V. (NASDAQ: RNA), the biopharmaceutical company focusing on RNA-modulating therapeutics for rare diseases with high unmet need, today announced that a comprehensive program of re-dosing has commenced, with the first patients now re-dosed in the United States. All dosing in the drisapersen clinical program had been placed on hold by GSK on September 20, 2013, upon announcement of the DEMAND III study results.

The re-dosing program in North America will include up to 72 patients across 14 sites who had participated in the drisapersen DEMAND V (Phase II) & DEMAND III (Phase III) studies. Prosensa regained the rights to drisapersen from its previous partner GSK in January 2014, and soon thereafter committed to re-dose patients as quickly as possible, accepting that the transfer of data and clinical materials, and the re-engagement with clinical sites would be a complex process.

"We are very pleased that we have been able to keep our promise to commence re-dosing patients with drisapersen in the third quarter of this year, which is taking a staged approach," said Dr. Giles Campion, Prosensa's Chief Medical Officer. "While we know the wait has been incredibly difficult for the boys (and their families), we have been working diligently on this achievement since regaining the rights to drisapersen and are grateful to all that have helped us in attaining this goal. While today is a major milestone for the Company, it is an even greater one for these first patients and their families, who have been anxiously awaiting drisapersen treatment since dosing was stopped almost one year ago" he added.

Drisapersen is administered subcutaneously, with once weekly dosing. Prosensa is aware of the burden that clinical trials can place on the families, and in this protocol efforts have been made to alleviate burden and facilitate participation.

Prosensa aims to enable home-dosing where feasible, and cost-free transport with straightforward logistics for clinical centers, through collaboration with Greenphire and the Medical Research Network (MRN).

Greenphire is the industry's leading provider of clinical payment technology and has made its proprietary ClinCard System available for patients in the US and Canada. The ClinCard system with Travel Module will help Prosensa manage the complex travel arrangements required for the patients involved in this study,

which will maintain a truly patient-centric environment. Patients, along with their caregivers and family members, can make use of Greenphire's expert travel services to arrange travel for expected study visits with minimal out-of-pocket costs. Similarly, Greenphire's logistical support allows clinician investigators to focus their time and attention where it's needed: patient care and study execution.

MRN is the world's leading provider of home healthcare for patients in clinical trials and will be making home-dosing options available to patients in the study.

"Given the weekly dosing regimen for drisapersen and the travel time and costs associated with these, we are pleased to collaborate with both Greenphire and MRN to ease the burden of this process to patients and their families, said Hans Schikan, Prosensa's Chief Executive Officer. "While making treatment options available for DMD patients globally is our number one priority, we also want to do this in a manner that is as seamless as possible for patients and their families."

Prosensa is also in in preparations for similar programs of re-dosing and support for all previously treated drisapersen patients.

In June, Prosensa announced that the United States Food and Drug Administration (FDA) outlined a regulatory path forward for drisapersen, under an accelerated approval pathway, based upon existing data, including the DEMAND II study, which was recently published in the Lancet Neurology. Prosensa remains on track to pursuing regulatory filings for drisapersen, initially in the US and Europe, with an FDA submission planned before the end of the year and an EMA submission shortly thereafter.

About Prosensa Holding N.V.

Prosensa (NASDAQ: RNA) is a biotechnology company engaged in the discovery and development of RNA-modulating therapeutics for the treatment of genetic disorders. Its primary focus is on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including Duchenne muscular dystrophy (DMD), Myotonic dystrophy and Huntington's disease.

Prosensa's current portfolio includes six compounds for the treatment of DMD, all of which have received orphan drug status in the United States and the European Union. The compounds use an innovative technique called exon-skipping to provide a personalized medicine approach to treat different populations of DMD patients. www.prosensa.com

About DMD

Duchenne muscular dystrophy (DMD) is a severely debilitating childhood neuromuscular disease that affects up to 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 the drisapersen clinical program

Drisapersen induces exon 51 skipping in the dystrophin gene and is intended for up to approximately 13% of all DMD patients. Drisapersen has been granted orphan designation in the European Union, the United States and Japan, and Breakthrough Therapy Designation in the United States.

The overall drisapersen clinical program comprises three double-blind, placebo-controlled studies (DEMANDII/DMD114117, DEMANDV/DMD114876 and DEMANDIII/DMD114044) and two long term open-label extension studies (DMD114673 and DEMAND IV/DMD114349). Over 300 patients have participated in clinical studies of drisapersen at more than 50 trial sites in 25 countries.

About exon skipping

The dystrophin gene is the largest gene in the body, consisting of 79 exons. Exons are small segments 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 be processed 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, or containing, the fault and thereby correct the RNA processing, enabling the production of a novel, largely functional dystrophin protein. Prosensa's exon skipping technology was licensed from Leiden University Medical Center.

About Greenphire ClinCard System

Greenphire's ClinCard System with Travel Module will help Prosensa manage the complex travel arrangements required for the 72 patients involved in this study and to maintain a truly patient-centric environment. The ClinCard System is a configurable, global web-based technology enabling clinical research sponsors and sites to deliver patient reimbursements in real-time. Patients, along with their caregivers and family members, can make use of Greenphire's expert travel services to arrange travel for expected study visits with minimal out-of-pocket costs. Similarly, Greenphire's logistical support allows clinician investigators to focus their time and attention where it's needed: patient care and study execution.

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About the Medical Research Network (MRN)

Established in 2006, MRN is the world's leading provider of global home healthcare for patients in clinical trials. Headquartered in the UK and with a US office in Chicago, MRN is currently offering home healthcare services across 27 countries and continuing to expand. MRN specialise in complex home healthcare services, focusing heavily on IMP administration (injection/infusion), paediatrics, rare and orphan diseases, and truly global studies; all MRN services are built on robust processes, unique technology innovations, central pharmacy and logistics capability and qualified nursing networks. In 2013 MRN also launched a unique social media driven feasibility tool, MRN-RAPID. By utilising this technology patients can be located both geographically and online, then mapped against site locations and competitive trials, painting a realistic picture of where best to set up clinical trials.

For more information visit www.themrn.co.uk

Forward Looking Statement

This press release contains certain forward-looking statements. All statements, other than statements of historical facts, contained in this press release, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include statements around our lead exon-skipping compound, drisapersen and the regulatory review around this program. Actual results may differ materially from those projected or implied in such forward-looking statements. Such forward-looking information involves risks and uncertainties that could significantly affect expected results. These risks and uncertainties are discussed in the Company's SEC filings, including, but not limited to, the Company's Form 6-K's and the Company's Annual Report on Form 20-F. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing our views as of any subsequent date. While we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so, even if our views change.



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