March 17, 2014

Prosensa Announces 48-Week Data from a U.S. Phase II Placebo-Controlled Study of Drisapersen in 51 DMD Boys

Clinically meaningful improvement from 24-week treatment period was maintained for 24 weeks after drisapersen administration ceased

Chicago, IL, March 17, 2014 (GLOBE NEWSWIRE) -- Prosensa Holding N.V. (NASDAQ: RNA), the Dutch biopharmaceutical company focusing on RNA-modulating therapeutics for rare diseases with high unmet need, today reported encouraging 48-week data from its U.S.-based, Phase II placebo-controlled study (DMD114876 or DEMAND V) of its lead compound, drisapersen, for the treatment of Duchenne Muscular Dystrophy (DMD).

The results of this study indicate that, compared to placebo, boys in the higher-dose drisapersen group (6 mg/kg once weekly) experienced stabilization and even improvements in their muscle function and physical activity as measured by the six-minute walk test (6MWT) for the 24-week treatment phase and maintained this improvement during the 24-week follow-up period. Additionally, when evaluating the percent-predicted six-minute walk distance (6MWD), a clinically meaningful treatment difference of 5.2% was observed at week 24 and 4.8% at week 48.

Principal investigator, Craig M. McDonald, M.D., Professor and Chair of Physical Medicine & Rehabilitation and Professor of Pediatrics at the University of California, Davis School of Medicine, will report the 48 week results in a poster session (Abstract #50) today at the Muscular Dystrophy Association 2014 Clinical Conference in Chicago, Illinois (March 16-19).

"Given the severity of the disease and the lack of disease modifying treatment options available, the results of this important study support the use of drisapersen at a dose of 6 mg/kg once weekly in the treatment of boys with DMD eligible for exon 51 skipping" Dr. McDonald said. "The maintenance of the clinically meaningful treatment benefit in the 24-week follow-up phase is very encouraging evidence for the drug's ability to produce prolonged stabilization of disease and may indicate that, at the 6 mg/kg once weekly dose, the drug has a long term treatment effect that helps delay disease progression in younger, less severe boys."

The study included 51 boys with DMD who were at least five years old, still able to walk and stand up from the floor without help in less than 15 seconds. As previously reported, boys in the group who received a 6mg/kg dose of drisapersen each week for the 24-week treatment period show a 27.1 meter improvement in the 6MWT (including a 16.1 m increase from baseline) over the boys in the placebo group at the end of the treatment period (p=0.069), indicating a clinically meaningful outcome for the primary endpoint. This study compared 6mg/kg/week with 3mg/kg/week and placebo and was not statistically powered to show a significant difference between the arms. A clinically meaningful treatment difference of 27.9 m over placebo (p=0.177) was maintained for 24 weeks after drisapersen administration ceased. This includes an overall mean increase from baseline of 14.7 m. In the drisapersen 6 mg/kg/week group, an improvement was seen in the percent-predicted 6MWD of 5.2% (p=0.051) and 4.8% (p=0.154) when compared to placebo at weeks 24 and 48, respectively.

Drisapersen at weekly doses of 3 and 6 mg/kg/week was generally well tolerated, although the majority of subjects treated with drisapersen reported injection-site reactions (none severe or serious). Renal abnormalities were common and occurred both in the placebo and drisapersen groups.

"Our priority remains improving the lives and outcomes of boys afflicted with this devastating disease. We are encouraged by these results, and are actively continuing with the analysis of the total drisapersen data set, which includes 300 patients and combined data representing 450 patient years to put these results into context," said Hans Schikan, CEO of Prosensa. "As we reported earlier this year, initial findings from further analyses of the aggregate drisapersen data suggest that treating earlier in DMD and treating longer shows a delay in the progression of the disease."

A copy of the poster can be accessed (starting March 18) under "Events & Presentations" through the Investors & Media section of the Prosensa corporate website at www.prosensa.com.

About Prosensa Holding N.V.

Prosensa (NASDAQ: RNA) is an innovative biotechnology company engaged in the discovery and development of ribonucleic acid-modulating, or 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,

Myotonic dystrophy and Huntington's disease. Its clinical portfolio of RNA-based product candidates is focused on the treatment of Duchenne muscular dystrophy, or DMD. Each of its DMD compounds has been granted orphan drug status in the United States and the European Union. Its first product candidate, drisapersen, can address a variety of mutations in the dystrophin gene, such as a deletion of exon 50 or exons 48 to 50.

About DMD

DMD is one of the most prevalent rare genetic diseases globally affecting up to 1 in 3,500 boys and is invariably fatal. There is currently no approved disease-modifying therapy for DMD. The progressive muscle-wasting that characterizes this disease is caused by inadequate production of dystrophin, a protein necessary for muscle function, as a result of mutations in the dystrophin gene. The different mutations, which are mostly deletions of one or more exons, found in the dystrophin gene result in distinct sub-populations of DMD patients. Prosensa are designing product candidates to address several sub-populations using its platform technology.

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 its 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 its exon-skipping drug pipeline and financial position. 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 containing this press release and certain sections of the Company's Registration Statement on Form F-1. In addition, any forward-looking statements represent its views only as of today and should not be relied upon as representing its views as of any subsequent date. While Prosensa may elect to update these forward-looking statements at some point in the future, Prosensa specifically disclaim any obligation to do so, even if its views change.

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Source: Prosensa Holding N.V.