

BioMarin and Prosensa Holding N.V. Reach Agreement on Intended Public Offer for 100% of Prosensa's Outstanding Stock; Will Add Duchenne Muscular Dystrophy Products to Rare-Disease Portfolio

Acquisition of Prosensa provides near-term opportunity to commercialize, if approved, its exon-skipping drug candidate, drisapersen, for Duchenne muscular dystrophy (DMD)

Drisapersen is currently under a rolling review as part of a New Drug Application process and has Orphan, Fast Track and Breakthrough Therapy designation by the FDA

Drisapersen, a potential first-to-market and best-in-class product for treating a large population of patients with a rare, fatal genetic disease represents up to 10,000 DMD patients

Follow-on products leveraging Prosensa's same technology platform in the pipeline target an additional 35,000 DMD patients in BioMarin's commercial territories

Investor conference call to be held today, November 24, 2014 at 5am PST (8am EST)

SAN RAFAEL, Calif., Nov. 24, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) and Prosensa Holding N.V. (Nasdaq:RNA) today announced that they have entered into a definitive agreement in which BioMarin will offer to purchase all of the outstanding ordinary shares of Prosensa for \$17.75 per share, for a total up front consideration of approximately \$680 million. In addition, two approximately \$80 million contingent milestones are payable for the approval of drisapersen in the U.S. no later than May 15, 2016 and Europe no later than February 15, 2017, respectively.

"BioMarin is dedicated to the rare disease community, and the acquisition of Prosensa fits strategically with our mission of delivering therapies that address serious unmet medical needs," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are committed to working closely with regulatory authorities worldwide in bringing a potentially breakthrough therapy to patients with this devastating condition."

Mr. Bienaimé continued, "We will leverage our experience at developing rare disease therapies to achieve regulatory approvals and bring drisapersen to market as quickly as possible. Further, if we are successful in advancing drisapersen to early regulatory approvals, we believe this transaction would be accretive to operating and GAAP profitability in 2017."

Pat Furlong, President and Founder of Parent Project Muscular Dystrophy said, "BioMarin has a successful track record of developing new therapies for people with devastating disorders and for effectively collaborating with health authorities and patient communities. We look forward to working with BioMarin to bring new treatments to boys with Duchenne and other forms of muscular dystrophy."

Under the terms of the definitive agreement, BioMarin will offer to acquire all of Prosensa's issued and outstanding ordinary shares and all ordinary share equivalents in an all cash transaction for \$17.75 per share for an upfront purchase price of approximately \$680 million. Prosensa shareholders may also receive two regulatory milestone payments of approximately \$80 million for receiving approval in the U.S. no later than May 15, 2016 and in Europe no later than February 15, 2017, respectively. In addition, within 5 business days of signing the purchase agreement BioMarin will purchase from Prosensa a \$50 million convertible note. If the transaction fails to close for any reason, the note will automatically convert into 4,395,914 shares of Prosensa's stock.

The transaction is expected to be accounted for as a business combination. BioMarin will maintain operations at Prosensa's headquarters, based in Leiden, The Netherlands and integrate Prosensa personnel from that office.

The acquisition will provide BioMarin with worldwide rights to multiple orphan-drug candidates, including drisapersen, which is currently under rolling review as part of a New Drug Application (NDA) with the Food and Drug Administration. Prosensa's pipeline is comprised of several potential products that leverage their proprietary RNA-modulating technology platform for the treatment of various genotypes of Duchenne muscular dystrophy and other genetic disorders.

Hans Schikan, Chief Executive Officer of Prosensa added, "BioMarin has established itself as a leader in rare diseases, characterized by strong management, thorough execution, and a resounding commitment to patients in developing and commercializing treatments where there is a high unmet medical need. This transaction will enhance Prosensa's mission by bringing innovative therapies to patients across the world as quickly and

efficiently as possible. The deal also creates shareholder value by positioning Prosensa's strong portfolio of orphan drug candidates for future success with a prominent rare disease company that has the experience and dedication to bring drisapersen and our follow-on compounds to the hands of patients who desperately need them."

BioMarin will effect the transaction primarily through a tender offer for all of the issued and outstanding Prosensa ordinary shares (the "Offer") and expect to close in the first quarter of 2015. The commencement of the Offer will be subject to having obtained workers council advice, and the consummation of the Offer is subject to the satisfaction of customary closing conditions for a transaction of this nature, including the tender of at least 80% of the issued and outstanding Prosensa ordinary shares and the receipt of regulatory clearance. Following completion of the Offer, the Supervisory Board of Prosensa will consist of five individuals designated by BioMarin and two individuals who currently serve on the Supervisory Board of Prosensa, who will act as independent directors. The two independent directors will, in accordance with Dutch practice, act as independent supervisory directors to protect the interest of any minority shareholders until BioMarin utilizes certain available reorganization structures available under Dutch law to acquire full ownership of Prosensa's outstanding shares and/or its business. An Extraordinary General Meeting will be convened in connection with the Offer and to adopt, among other things, certain resolutions relating to the reorganization of Prosensa.

About Exon skipping

Synthetic oligonucleotides can bind to specific regions of pre-mRNA to induce exon skipping or exon inclusion, reduce mutated toxic RNA or protein, remove specific protein domains, or block protein expression. Exon skipping can restore the proper reading frame to the dystrophin mRNA, allowing shorter but still functional dystrophin protein to be produced, potentially maintaining or improving muscle function in people with DMD.

About Drisapersen

Drisapersen induces the skipping of dystrophin exon 51, potentially providing a therapeutic benefit to DMD patients for whom skipping of exon 51 restores the proper dystrophin reading frame, corresponding to approximately 13% of DMD patients. Drisapersen has been administered to over 300 patients in seven clinical studies. It is generally well tolerated, with an adverse event (AE) profile consistent with this class of molecule. Drisapersen is currently under rolling review as part of a New Drug Application (NDA) with the Food and Drug Administration.

About Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) is the most common fatal genetic disorder diagnosed in childhood, affecting approximately 1 in every 3,500 live male births (about 20,000 new cases each year). DMD is caused by a mutation in the gene that encodes for [dystrophin](#), a protein that is important in connecting the cytoskeleton of muscle fibers to the extracellular matrix. Its deficiency in DMD leads to progressive muscle weakness, loss of ambulation in early adolescence, and typically death due to pulmonary or cardiac insufficiency in the late twenties. Because the Duchenne gene is found on the X-chromosome, it primarily affects boys; however, it occurs across all races and cultures. There is currently no cure for DMD.

About Prosensa

Prosensa N.V. is a biotechnology company based in Leiden, The Netherlands, which discovers and develops RNA-modulating therapeutics for the treatment of genetic disorders. The primary focus is on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including DMD, myotonic dystrophy and Huntington's disease. Prosensa's lead compound for DMD, is drisapersen. Its seven follow-on programs for other DMD genotypes are currently in various stages of development from preclinical through Phase 2. In addition, the Company has preclinical products in development for Huntington's disease and myotonic dystrophy. The Company has 85 employees.

About Parent Project Muscular Dystrophy

[Duchenne](#) is a fatal genetic disorder that slowly robs young men of their muscle strength. [Parent Project Muscular Dystrophy \(PPMD\)](#) is the largest most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne muscular dystrophy, the mission is to end Duchenne.

PPMD invests deeply in treatments for this generation of young men affected by Duchenne and in research that will benefit future generations. They advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. PPMD demands optimal care, and strengthens, unite and educate the global Duchenne community.

Everything they do and everything they have done since their founding in 1994—helps boys with Duchenne live

longer, stronger lives. They will not rest until every young man has a treatment to end Duchenne. Go to www.ParentProjectMD.org for more information or to learn how you can support the efforts and help families affected.

Conference Call Details

BioMarin will host a conference call and live audio webcast today at 5am PST/8am EST to discuss the transaction. The live audio webcast will be available via the Investors and Media section of the BioMarin Pharmaceutical website at www.BMRN.com. Interested parties may now access the PowerPoint presentation that will be used for the call at the same URL. A replay will be available for one week following the call.

U.S. / Canada Dial-in Number: 877.303.6313

International Dial-in Number: 631.813.4734

Conference ID: 40640313

Replay Dial-in Number: 855.859.2056

Replay International Dial-in Number: 404.537.3406

Conference ID: 40640313

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include: Naglazyme® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (elosulfase alfa) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; talazoparib (BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 3 clinical development for the treatment of Pompe disease; BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia; and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1), which is currently in Phase 1 for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

BioMarin Forward Looking Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the possible purchase of Prosensa Holding N.V.; the development of drisapersen if the purchase closes; the potential of drisapersen if the purchase closes; and the potential market for drisapersen. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: possible competitive offers for the acquisition of Prosensa; actions by Prosensa's shareholders at the extraordinary general meeting of Prosensa and in the tender process; results and timing of current and planned preclinical studies and clinical trials of drisapersen; our ability to successfully manufacture drisapersen; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning drisapersen; the market for each the product; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the risk factors contained under the caption "Risk Factors" in BioMarin's 2013 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

Additional Information

The Offer described in this communication has not yet commenced, and this communication is for informational purposes only and is neither an offer to purchase nor a solicitation of an offer to sell any ordinary shares of Prosensa or any other securities. On the commencement date of the Offer, a tender offer statement on Schedule TO, including an offer to purchase, a letter of transmittal and related documents, will be filed with the United States Securities and Exchange Commission (the "SEC"). Thereafter, Prosensa will file a solicitation/recommendation statement on 14D-9 with the SEC. The solicitation and offer to purchase ordinary shares of Prosensa will only be made pursuant to the offer to purchase, the letter of transmittal and related documents filed as a part of the Schedule TO.

Investors and security holders of Prosensa are urged to read both the schedule to (and the included offer to purchase) and the solicitation/recommendation statement, as they may be amended from time to time and other relevant documents filed with the SEC when they become available before they make any decision with respect to the tender offer, because they will contain important information about the terms of the offer, the proposed transactions and the parties thereto.

Investors and security holders may obtain a free copy of these statements (when available) and other documents filed with the SEC at the website maintained by the SEC at www.sec.gov or by directing such requests to the Information Agent for the tender offer that will be named in the tender offer statement.

Prosensa Cautionary Statement Regarding Forward-Looking Statements

Some of the statements contained in this communication are forward-looking statements, including statements regarding the expected consummation of the transaction, which involves a number of risks and uncertainties, including the satisfaction of closing conditions for the acquisition, such as regulatory approval for the transaction, and the tender of at least eighty percent of the outstanding ordinary shares of the company, the possibility that the transaction will not be completed and other risks and uncertainties discussed in the Prosensa's public filings with the SEC, including the "Risk Factors" section of Prosensa's annual report on Form 20-F for the year ended December 31, 2013, as well as the tender offer documents to be filed by BioMarin and the solicitation/recommendation statement to be filed by Prosensa. These statements are based on current expectations, assumptions, estimates and projections, and involve known and unknown risks, uncertainties and other factors that may cause results, levels of activity, performance or achievements to be materially different from any future statements. These statements are generally identified by words or phrases such as "believe", "anticipate", "expect", "intend", "plan", "will", "may", "should", "estimate", "predict", "potential", "continue," or the negative of such terms or other similar expressions. If underlying assumptions prove inaccurate or unknown risks or uncertainties materialize, actual results and the timing of events may differ materially from the results and/or timing discussed in the forward-looking statements, and you should not place undue reliance on these statements. Prosensa disclaims any intent or obligation to update any forward-looking statements as a result of developments occurring after the period covered by this report or otherwise.

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