Poised for Significant Growth and Profitability, BioMarin Shares Company Highlights During R&D Day on November 14th in New York

Valoctocogene Roxaparvovec on Track for US and EU Regulatory Submissions by Year End

Vosoritide Sustains Height Gain in Phase 2 Study, Phase 3 Topline Results Expected by Year End

Next Two IND Candidates Identified: 3rd Gene Therapy Candidate in Hereditary Angioedema, Potential 2nd Indication for Vosoritide in Dominantly Inherited Short Stature

Guidance of GAAP Net Income Break-Even or Better in 2020, If Valoctocogene Roxaparvovec Commercially Launched in 2H 2020

SAN RAFAEL, Calif., Nov. 14, 2019 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) updated the investment community on the Company's research and development portfolio, which is focused on innovative therapies to treat rare diseases.

"BioMarin is at an inflection point where our combined understanding of the biology of many rare diseases and key technologies like gene therapy position us not only for exceptional growth over the next decade, but for GAAP profitability in 2020," said JJ Bienaimé, Chairman and CEO of BioMarin. "We are excited about the possibilities of our two late-stage clinical programs in hemophilia A and achondroplasia potentially to transform the course of these conditions. We are also looking forward to leveraging our experience in gene therapy and genetic disease to bring a rich pipeline of new investigational therapies into the clinic."

Valoctocogene Roxaparvovec, Investigational Gene Therapy for Severe Hemophilia A

BioMarin is on track to submit marketing applications for investigational gene therapy, valoctocogene roxaparvovec, to health authorities in the United States and Europe before year end.

"Valoctocogene roxaparvovec shows a transformational reduction in bleeding and need for Factor VIII infusions. We remain confident in its long-term efficacy and durability based on our newly presented liver biopsy data that demonstrates that the DNA transferred by the gene therapy vector remains detectable in hepatocytes years after the one-time infusion. We are also pleased to see that the material used in Phase 3 produces comparable amounts of stable vector in blood samples as was observed in Phase 2," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "Valoctocogene roxaparvovec has the most data over the longest period of time of any gene therapy in hemophilia A, and we are looking forward to providing a further update with four years of data in the Phase 2 study in mid-2020."

BioMarin noted that the Phase 3 study has now dosed 130 study participants, and that the Company completed its process performance qualification (PPQ) campaign, which was the last step in the Chemistry, Manufacturing and Controls package (CMC) package of the regulatory submissions.

Valoctocogene roxaparvovec has been accepted for PRIME scheme from the European Medicines Agency (EMA). In addition, the EMA recently granted BioMarin's request for accelerated assessment of valoctocogene roxaparvovec, for adults with severe hemophilia A. Additionally, the FDA has granted valoctocogene roxaparvovec Breakthrough Therapy designation. Valoctocogene roxaparvovec has Orphan Drug designation from the FDA and

the EMA.

Vosoritide for Achondroplasia

The Company provided an update on its clinical program for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of disproportionate short stature in humans at its annual R&D Day. An ongoing, open-label, Dose Finding Phase 2 study of vosoritide for achondroplasia demonstrated over 54 months that children in cohort 3 (N=10) of the study, at a dose of 15 µg/kg/day, achieved a statistically significant ($p < 0.005$) cumulative mean additional height gain of 9.0 cm compared to children, matched for age and gender, in a new natural history achondroplasia dataset (N=619). 2.2 cm of this additional increase occurred in the last 12 months further informing our understanding of vosoritide's ongoing treatment impact.

In addition, BioMarin reaffirmed that it plans to provide top-line data on the randomized, placebo-controlled Phase 3 study by the end of the year.

Early Stage Pipeline

BioMarin has identified its next two investigational new drug (IND) candidates to enter into its pipeline. The first will be a third gene therapy, BMN 331, for Hereditary Angioedema (HAE). The Company will build on its ever wider and deeper experience in developing gene therapies for hemophilia A and phenylketonuria (PKU) to improve efficiencies in the development process,and optimize capsid and transgene design.

The second IND will investigate a new indication for vosoritide in broader genetic statural deficiencies starting with dominantly inherited short stature

(DISS). The Company will build on its learnings with vosoritide in achondroplasia and look for efficiencies in the development process, particularly around pre-clinical research and manufacturing.

BMN 307, Pre-Clinical Gene Therapy for Phenylketonuria (PKU)

In September 2019, BioMarin filed a clinical trial application (CTA) for BMN 307 in the United Kingdom. BMN 307 research represents a potential third Phenylketonuria (PKU) treatment option in BioMarin's PKU franchise. BMN 307 is one of three gene therapies in the Company's pipeline.

BMN 307 is an investigational AAV5-phenylalanine hydroxylase (PAH) gene therapy designed to reduce blood phenylalanine (Phe) concentrations levels in patients with PKU. After preclinical experiments in mice with PKU showed rapid, complete and lifelong normalization of Phe in mice with PKU, BMN 307 will be evaluated to determine whether a single dose of treatment can restore Phe metabolism in patients with PKU, normalize plasma Phe level, and enable a normal diet. The company expects to start enrolling patients in a Phase 1/2 trial early next year and is actively preparing regulatory submissions for other countries.

GAAP Net Income Break-Even or Better in 2020

BioMarin also indicated that the Company expects GAAP net income to be break-even or better in 2020, assuming a commercial launch of valoctocogene roxaparvovec in the second half of 2020.

About BioMarin and Disease Information

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-

rare genetic diseases. The Company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com. Information on such website is not incorporated by reference into this press release.

About Achondroplasia

Achondroplasia, the most common form of disproportionate short stature in humans, is characterized by slowing of endochondral ossification, which results in disproportionate short stature and disordered architecture in the long bones, spine, face and base of the skull. This condition is caused by a mutation in the fibroblast growth factor receptor 3 gene (FGFR3), a negative regulator of bone growth. Beyond disproportionate short stature, people with achondroplasia can experience serious health complications, including foramen magnum compression, sleep apnea, bowed legs, mid-face hypoplasia, permanent sway of the lower back, spinal stenosis and recurrent ear infections. Some of these complications can result in the need for invasive surgeries such as spinal cord decompression and straightening of bowed legs. In addition, studies show increased mortality at every age.

More than 80% of children with achondroplasia have parents of average stature and have the condition as the result of a spontaneous gene mutation. The worldwide incidence rate of achondroplasia is about one in 25,000 live births. Vosoritide is being tested in children whose growth plates are still "open", typically those under 18 years of age. This is approximately 25% of people with achondroplasia. In the U.S., Europe, Latin America and the Middle East, there are currently no licensed medicines for achondroplasia.

Vosoritide Safety

Vosoritide, administered in over 28,000 injections, was generally well tolerated at all doses. The majority of adverse events (AEs) were mild and no serious adverse events (SAEs) were reported as study drug-related. Across all doses, injection site reactions and hypotension were the most common drug-related AEs. All injection site reaction events were transient. AEs of hypotension were mild, transient and resolved without medical intervention, and the majority were asymptomatic and reported in context of routine blood pressure measurements. No new safety findings were observed at the 30 µg/kg/day dose.

About Hemophilia A

Hemophilia A, also called factor VIII (FVIII) deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. Although it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous mutation, a new mutation that was not inherited. Approximately 1 in 10,000 people is born with Hemophilia A. People living with the disease are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their lives. People with severe hemophilia often bleed spontaneously into their muscles or joints. The standard of care for the 43% of individuals with hemophilia A who are severely affected is a prophylactic regimen of Factor VIII infusions two to three times per week. Even with prophylactic regimens, many people still experience spontaneous bleeding events that result in progressive and debilitating joint damage.

Valoctocogene Roxaparvovec Phase 1/2 Study Safety

Overall, valoctocogene roxaparvovec has been well-tolerated by patients across all doses. No patients developed inhibitors to Factor VIII and no patients withdrew from the study. The most common AEs across all dose cohorts were alanine aminotransferase (ALT) elevation (11 patients, 73%);

arthralgia, aspartate aminotransferase elevation, and headache (7 patients each, 47%); back pain and fatigue (5 patients each, 33%). Two patients reported SAEs during the study. One patient was hospitalized for observation after developing Grade 2 pyrexia with myalgia and headache within 24 hours of receiving valoctocogene roxaparvovec. The event resolved within 48 hours following treatment with paracetamol, an over-the-counter treatment for pain and fever. The event was assessed as related to valoctocogene roxaparvovec. The other AE was assessed as not related to the therapy, attributed to a planned knee surgery to treat hemophilic arthropathy, and Grade 1 in severity. No complications were reported.

About Hereditary Angioedema

Hereditary angioedema (HAE) is a rare and potentially life-threatening genetic condition that occurs in about 1 in 10,000 to 1 in 50,000 people. HAE patients have a defect in the gene that controls a blood protein called C1 Inhibitor. The genetic defect results in production of either inadequate or non-functioning C1-Inhibitor protein. Normal C1-Inhibitor helps to regulate the complex biochemical interactions of blood-based systems involved in inflammatory responses. Because defective C1-Inhibitor does not adequately perform its regulatory function, a biochemical imbalance can occur and produce unwanted peptides that induce the capillaries to release fluids into surrounding tissue, thereby causing edema.

HAE symptoms include episodes of edema (swelling) in various body parts including the hands, feet, face and airway. In addition, patients often have bouts of excruciating abdominal pain, nausea and vomiting that is caused by swelling in the intestinal wall. Airway swelling is particularly dangerous and can lead to death by asphyxiation. *[Source last checked on 11-13-19]*

About Dominantly Inherited Short Stature

People affected by dominantly inherited short stature (DISS) have one inherited mutation from one parent that is the cause of short stature. It is believed to be a subgroup of ISS. In DISS cases, it is thought that the CNP may be capable of signaling and promoting growth.

A full replay of the R&D Day presentation can be found at BioMarin.com.

Forward Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about its development programs and regulatory actions related to these programs, including the timing of (i) the filing of regulatory submissions for valoctocogene roxaparvovec, the decisions by regulators regarding BioMarin's regulatory submissions for valoctocogene roxaparvovec and the anticipated release of four years of data in the Phase 2 study in mid-2020, (ii) BioMarin's preclinical studies and clinical studies and trials, (iii) completion of enrollment of those studies and trials, and (iv) announcements of data from those studies and trials, including the timing of the release of Phase 3 topline results for vosoritide; the sustained height growth in vosoritide; the continued clinical development and commercialization of BioMarin's commercial products and product candidates; the possible approval and commercialization of BioMarin's product candidates; and statements about expectation of GAAP net income break-even or better in 2020 if valoctocogene roxaparvovec is commercially launched in the second half 2020. 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: results and timing of current and planned preclinical studies and clinical trials of our product candidates, the continued

clinical experiences of the patients in the current clinical studies; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities; our ability to successfully manufacture our product candidates for the preclinical and clinical trials; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the quarter ended September 30, 2019 and future SEC filings and reports by BioMarin. BioMarin undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

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