BioMarin Announces the Ministry of Health, Labor and Welfare (MHLW) in Japan Granted Approval for VOXZOGO® (vosoritide) for Injection for the Treatment of Children with Achondroplasia, Whose Growth Plates are Not Closed

No Lower Age Restriction for Treatment

Japan Accounts for Approximately Half of the 1,500 Patient Opportunity in APAC Region

SAN RAFAEL, Calif., June 21, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that the Ministry of Health, Labor and Welfare (MHLW) in Japan granted approval of the registration of VOXZOGO® (vosoritide) for injection, indicated for the treatment of achondroplasia in children of all ages, whose growth plates are not closed. Voxzogo, a modified C-type natriuretic peptide (CNP), directly targets the underlying pathophysiology of achondroplasia by down regulating fibroblast growth factor receptor 3 (FGFR3) signaling and consequently promoting endochondral bone formation.

BioMarin Announces Approval of VOXZOGO in Japan for Children of all ages

"We are delighted to offer children in Japan of all ages with achondroplasia access to a treatment option that addresses the underlying genetic mechanism of the condition," said Jean-Jacques Bienaimé, Chairman and CEO of BioMarin. "CNP was discovered as a natural regulator of bone growth in Japan in 1990 so we are especially proud to be able to offer a therapeutic choice there. We look forward to nurturing our partnerships with advocates and the achondroplasia community in Japan and beyond."

The MHLW in Japan based its decision on the outcomes of a global Phase 3 randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of Voxzogo and the long-term extension of this Phase 3 study as well as data from patients participating in a Phase 2 randomized, double-Blind, placebo-controlled clinical trial evaluating the safety and efficacy of Voxzogo in infants and young children with achondroplasia, age 0 to < 60 months.

In 2021, Voxzogo received approvals in the United States, Europe and Brazil.

About Achondroplasia

Achondroplasia, the most common form of skeletal dysplasia leading to disproportionate short stature, is characterized by slowing of endochondral bone growth, 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 gain of function mutation in the fibroblast growth factor receptor 3 gene (*FGFR3*), a negative regulator of bone growth. More than 80% of children with achondroplasia have parents of average stature and have the condition as the result of a spontaneous change in the gene.

Around 25% of individuals living with achondroplasia have open growth plates. The worldwide incidence rate of achondroplasia is about one in 25,000 live births.

VOXZOGO Safety

Safety and efficacy of Voxzogo in patients with achondroplasia were assessed in one 52–week, multi–center, randomized, double–blind, placebo–controlled, Phase 3 study. Transient decreases in blood pressure have been observed with Voxzogo. In the clinical study, 8 (13%) of 60 patients treated with Voxzogo had a total of 11 events of transient decreases in blood pressure compared to 3

(5%) of 61 patients on placebo, over a 52-week treatment period. Patients with significant cardiac or vascular disease or on anti-hypertensive medicine were excluded from the trial. To reduce the risk of a decrease in blood pressure and associated symptoms (dizziness, fatigue and/or nausea), patients should be well hydrated and have adequate food intake prior to administration.

The most common adverse reactions, occurring in greater than or equal to 5% of patients treated with Voxzogo and at a percentage greater than placebo in the Phase 3 study are injection site reactions (including redness, itching, swelling, bruising, rash, hives, pain), vomiting, joint pain, decreased blood pressure, gastroenteritis, diarrhea, dizziness, ear pain, influenza, fatigue, seasonal allergy, and dry skin.

U.S. VOXZOGO Indication

In the United States, VOXZOGO is a prescription medicine used to increase linear growth in children with achondroplasia who are 5 years of age and older with open growth plates (epiphyses). VOXZOGO is approved under accelerated approval based on an improvement in annualized growth velocity. Continued approval may be contingent upon verification and description of clinical benefit in confirmatory trials.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare genetic diseases. The company's portfolio consists of seven 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.

Forward-Looking Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: BioMarin's VOXZOGO development program generally, the potential market size in Japan, and APAC, and that the continued approval for this indication in the U.S. may be contingent upon the verification and description of clinical benefit in confirmatory studies. 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: the results and timing of ongoing and possible future clinical trials of VOXZOGO; our ability to successfully manufacture Voxzogo; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning Voxzogo; the actual size of the Japanese market for VOXZOGO 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 March 31, 2022 as such factors may be updated by any subsequent reports. 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.

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