BioMarin Announces Favorable Results from Global Phase 2 Study of VOXZOGO™ (vosoritide) for Injection in Infants and Young Children with Achondroplasia at The Endocrine Society Annual Meeting, ENDO 2022 (June 11-14), in Atlanta

Improvement in Height Z-Score and Annualized Growth Velocity Observed, Consistent with Children Over 5

Interactions with Health Authorities Planned for H2 2022 to Discuss Expanding Access to Younger Children

SAN RAFAEL, Calif., June 13, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) presented data at The Endocrine Society Annual Meeting, ENDO 2022, demonstrating the Company's ongoing commitment to understanding the lifetime impact of achondroplasia, the most common form of disproportionate short stature. The Company provided data from a Phase 2 randomized, double-blind, placebo-controlled clinical trial to evaluate the safety and efficacy of VOXZOGO™ (vosoritide) for Injection in infants and children aged 0 to less than five years old.

In the overall population of all randomized and sentinel subjects, the study showed an improvement in height Z-score (a measure of height adjusted for age and sex in reference to the average stature population and reported as a Standard Deviation, SD) as compared to placebo at 52 weeks. Voxzogo (n=43), compared to placebo (n=32), increased height Z-score by 0.30 SD (95% CI 0.07, 0.54) and annualized growth velocity (AGV) by 0.92cm/year (95% CI 0.24, 1.59). This improvement in height Z-score was consistent with improvements previously observed after one year of treatment in children over five years of age. In the randomized population (excluding sentinel subjects)

and in the individual age subgroups, a trend toward increased height Z-score was observed. Voxzogo did not significantly impact upper-to-lower body segment ratio, which changed by -0.06 (95% CI -0.15, 0.03) over this 52-week period.

"We are pleased by these 52-week analysis results showing positive changes with Voxzogo compared to placebo in height Z-score and AGV," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "We look forward to discussing next steps regarding our efforts to expand access to Voxzogo treatment for this younger age group. We remain grateful to the children and families participating in the clinical study program."

"These data are very encouraging and supportive of early treatment initiation – there is clearly the potential these younger children could see meaningful benefit from treatment with Voxzogo. Importantly this is just the beginning: we look forward to the results of future analyses addressing additional endpoints and await further data on longer term follow up in this population with complex needs," said Melita Irving, Clinical Geneticist at Guy's and St Thomas' NHS Foundation Trust, London, UK and investigator for the Voxzogo clinical program at the Evelina London Children's Hospital.

BioMarin intends to meet with regulatory health authorities in the second half of 2022 to discuss next steps to expand access to Voxzogo for the treatment of achondroplasia in younger children.

Voxzogo Safety

The safety profile was generally consistent with that seen in older subjects in the Phase 3 Voxzogo 301 study (the current label population). Serious Adverse Events (SAEs) were higher in the placebo group (18%) compared to Voxzogo-

treated children (7%). All SAEs, including a fatal respiratory arrest (reported as a sudden infant death syndrome in a treated infant with pre-existing respiratory morbidity), were deemed by the investigators to be unrelated to treatment. The most common adverse events were mild and self-limiting injection site reactions. See the U.S. Important Safety Information below and full Prescribing Information and Patient Prescribing Information for additional safety information.

Study Description

The 52-week phase 2 study enrolled 75 infants and young children with achondroplasia, aged zero to less than five years old (60 months). The study consists of three cohorts by age (24 months to less than 5 years, 6 months to less than 24 months, less than 6 months) and is followed by a subsequent open-label extension trial where all children receive active treatment. Children in this study will have completed a minimum three- or six-month baseline study to determine their respective baseline growth prior to entering the Phase 2 study. The objectives of the study are to evaluate safety, tolerability, and the effect of Voxzogo on growth. The study also evaluated proportionality, functionality, quality of life, sleep apnea, and foramen magnum dimension, as well as the advent of major illnesses and surgeries which frequently occur in children with achondroplasia.

About VOXZOGO™ (vosoritide) for Injection

In patients with achondroplasia, endochondral bone growth, an essential process by which bone tissue is created, is negatively regulated due to a gain of function mutation in fibroblast growth factor receptor 3 gene (*FGFR3*). Voxzogo, a C-type natriuretic peptide (CNP) analog, represents a new class of therapy, which acts as a positive regulator of the signaling pathway downstream of FGFR3 to promote endochondral bone growth.

Voxzogo continues to be studied in a broad clinical development program in achondroplasia, and safety and efficacy are being further evaluated across different ages and over time. To date, 250 children with achondroplasia from eight countries have been enrolled in seven BioMarin clinical studies evaluating the safety and efficacy of Voxzogo.

Voxzogo is approved in the U.S. and indicated to increase linear growth in pediatric patients with achondroplasia who are 5 years of age and older with open epiphyses. This indication is approved under accelerated approval based on an improvement in annualized growth velocity. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).To fulfill this post-marketing requirement, BioMarin intends to use the ongoing open-label extension studies compared to available natural history.

Voxzogo is also approved in the EU and Brazil in children with achondroplasia who are 2 years of age and older with open growth plates. Marketing authorization reviews are in progress in Japan and Australia with potential approvals in these countries in 2022.

About Achondroplasia

Achondroplasia, the most common form of skeletal dysplasia leading to 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 change 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. Voxzogo is being tested in children whose growth plates are still "open", typically those under 18 years of age. Approximately 25% of people with achondroplasia fall into this category.

VOXZOGO U.S. Important Safety Information

What is VOXZOGO used for?

- 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).
- It is not known if VOXZOGO is safe and effective in children with achondroplasia under 5 years of age.
- 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.

What is the most important safety information about VOXZOGO?

- VOXZOGO may cause serious side effects including a temporary decrease in blood pressure in some patients. To reduce the risk of a decrease in blood pressure and associated symptoms (dizziness, feeling tired, or nausea), patients should eat a meal and drink 8 to 10 ounces of fluid within 1 hour before receiving VOXZOGO.

What are the most common side effects of VOXZOGO?

- The most common side effects of VOXZOGO include injection site reactions (including redness, itching, swelling, bruising, rash, hives, and injection site pain), vomiting, joint pain, decreased blood pressure, and stomachache. These are not all the possible side effects of VOXZOGO. Ask your healthcare provider for medical advice about side effects, and about any side effects that bother the patient or that do not go away.

How is VOXZOGO taken?

- VOXZOGO is taken daily as an injection given under the skin, administered by a caregiver after a healthcare provider determines the caregiver is able to administer VOXZOGO. Do not try to inject VOXZOGO until you have been shown the right way by your healthcare provider. VOXZOGO is supplied with Instructions for Use that describe the steps for preparing, injecting, and disposing VOXZOGO. Caregivers should review the Instructions for Use for guidance and any time they receive a refill of VOXZOGO in case any changes have been made.
- Inject VOXZOGO 1 time every day, at about the same time each day. If a dose of VOXZOGO is missed, it can be given within 12 hours from the missed dose. After 12 hours, skip the missed dose and administer

the next daily dose as usual.

- The dose of VOXZOGO is based on body weight. Your healthcare provider will adjust the dose based on changes in weight following regular check-ups.
- Your healthcare provider will monitor the patient's growth and tell you when to stop taking VOXZOGO if they determine the patient is no longer able to grow. Stop administering VOXZOGO if instructed by your healthcare provider.

What should you tell the doctor before or during taking VOXZOGO?

- Tell your doctor about all of the patient's medical conditions including
    - If the patient has heart disease (cardiac or vascular disease), or if the patient is on blood pressure medicine (anti-hypertensive medicine).
    - If the patient has kidney problems or renal impairment.
    - If the patient is pregnant or plans to become pregnant. It is not known if VOXZOGO will harm the unborn baby.
    - If the patient is breastfeeding or plans to breastfeed. It is not known if VOXZOGO passes into breast milk.

- Tell your doctor about all of the medicines the patient takes, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

You may report side effects to BioMarin at 1-866-906-6100. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see additional safety information in the full Prescribing Information and Patient Information.

Patient Support for Accessing Voxzogo

To reach a BioMarin RareConnections® case manager, please call, toll-free, 1-866-906-6100 or e-mail support@biomarin-rareconnections.com. For more information about Voxzogo, please visit www.voxzogo.com. For additional information regarding this product, please contact BioMarin Medical Information at medinfo@bmrn.com.

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 benefits of Voxzogo for infants and young children; the continued clinical development of Voxzogo, including, without limitation, the timing, design, conduct, and results of the global Phase 2 Study of Voxzogo for infants and young Children with achondroplasia aged 0 to less than five years old; BioMarin intention's to meet with regulatory health authorities in the second half of 2022 to discuss next steps to expand access to Voxzogo for the

treatment of achondroplasia in younger children; BioMarin's intention to use the ongoing open-label extension studies of Voxzogo compared to available natural history to fulfill the Company's post-marketing requirement for Voxzogo's continued approval in the U.S.; and the potential actions of regulatory authorities regarding the Company's marketing authorization applications for Voxzogo, including, without limitation, the Company's applications in Japan and Australia. 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 clinical trials of Voxzogo; our ability to enroll participants into such clinical trials, 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; 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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