Update for Achondroplasia Associations

Impact of the COVID-19 pandemic

We are providing this statement to provide general information about the BioMarin clinical development program for achondroplasia in the context of the COVID-19 (Coronavirus) pandemic.

As more cases of COVID-19 are confirmed, we recognize the increased challenges and concerns faced by participants in the BioMarin clinical studies. The safety and well-being of study participants, healthcare providers, and our communities is paramount as the ongoing COVID-19 pandemic impacts the health and livelihoods of many worldwide.

Many regulatory bodies, health authorities and government departments have now issued directives and guidance to help sponsors safely and appropriately manage clinical studies during this pandemic. BioMarin continues to conduct our studies according to this guidance. BioMarin plans regulatory submissions of Vosoritide in 3Q 2020 in both US and Europe. Those regulatory submissions will be reviewed by regulators over many months to allow them to evaluate Vosoritide's safety and efficacy in order to determine whether it can be made available.

BioMarin is in regular contact with investigators and study site staff at sites in all countries and is providing guidance to local study teams regarding study conduct. We acknowledge and are extremely grateful for all study participants and staff for their contribution and commitment to this program, especially during this pandemic.

> For more information: Individuals enrolled in any BioMarin clinical study should contact their trial site staff for the latest updates and to answer any specific questions they might have.

This is a rapidly evolving situation and all efforts are being made to continue the BioMarin clinical program while remaining acutely aware of the safety for all individuals. We are committed to partnering with the community to continually reassess, find solutions and provide necessary updates.

For additional information on BioMarin clinical studies:

- Visit www.clinicaltrials.gov and type in the study code BMN 111
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information toll free at 1-800-983-4587 or medinfo@bmrn.com

Update for Achondroplasia Associations

BioMarin is a global pharmaceutical company with more than 20 years of experience in developing medicines for rare genetic conditions.

We are in the advanced stages of developing an investigational medicine called vosoritide, for achondroplasia. An investigational medicine is a drug that is being studied to see if it is safe and effective to treat a particular condition. The results of these studies will be reviewed by regulators who will then decide whether to approve the drug to be marketed. BioMarin plans to submit the data from these studies to regulatory authorities in 2020.

Over 500 children with achondroplasia from 8 countries have enrolled in BioMarin clinical studies. These children and their families have been crucial to the ongoing research into the safety and efficacy of vosoritide. We are incredibly grateful to everyone who participates in our clinical studies.

Ongoing studies

111-901: an observational study

111-901 will be open for enrollment until 2020. The study does not involve an investigational medicine.

Data from this study will be compared to data from BioMarin studies that treat participants with vosoritide to better understand the investigational medicine's effects.

For more information on 111-901, please visit:
https://clinicaltrials.gov/ct2/show/NCT01603095

111–206 and 111-208: clinical studies

111–206 and 111–208 will be open for enrollment through 2020. The 111-206 study will last for one year and the 111-208 study will last longer than one year. These clinical studies are designed to study vosoritide's safety and effect on participants' growth, need for surgeries, bone health and quality of life.

(continued)

The study will also measure vosoritide's effect on participants':

- Proportionality of body segments and limbs
- Leg bowing, elbow extension, and arm span
- Hip function and joint pain
- Sleep apnea
- Health-related quality of life and ability to perform day-to-day activities

For more information on 111-206, please visit:
https://clinicaltrials.gov/ct2/show/NCT03583697

For more information on 111-208, please visit:
https://clinicaltrials.gov/ct2/show/NCT03989947

111-501 and 111-502: observational studies on lifetime impact

111-501 (LIAISE) is open for enrollment until the end of 2019, and 111-502 (LISA) is open for enrollment until June 2020. The study will take less than a day and does not involve an investigational medicine.

These studies aim to better understand what it is like to live with achondroplasia by finding health trends from childhood through to adulthood. This information may eventually result in better care.

For more information on 111-501 (LIAISE), please visit:
https://clinicaltrials.gov/ct2/show/NCT03449368

For more information on 111-502 (LISA), please visit:
https://clinicaltrials.gov/ct2/show/NCT03872531

Ongoing Studies, Enrollment Complete

111-301 and 111-302: clinical studies

Enrollment for these phase 3 studies is now complete and BioMarin has announced their primary endpoint results. The company will present more detailed information at an upcoming clinical meeting.

These clinical studies are designed to study vosoritide's safety and effect on participants' growth, need for surgeries, bone health and quality of life.

The study will also measure vosoritide's effect on participants':

- Proportionality of body segments and limbs
- Leg bowing, elbow extension, and arm span

(continued)

- Major illnesses
- Health-related quality of life and ability to perform day-to-day activities

For more information on 111-301, please visit:
https://clinicaltrials.gov/show/NCT03197766

For more information on 111-302, please visit:
https://clinicaltrials.gov/ct2/show/NCT03424018

111-202 and 111-205: dose finding and extension study

BioMarin has completed the dose evaluation study 111-202 and is currently following all participants in the long-term extension study called 111-205.

- All participants are receiving the investigational medicine vosoritide

For more information on 111-202, please visit:
https://clinicaltrials.gov/ct2/show/NCT02055157

For more information on 111-205, please visit:
https://clinicaltrials.gov/ct2/show/NCT02724228

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