Update for Achondroplasia Associations

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

BioMarin has announced the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) and a New Drug Application (NDA) to the US Food and Drug Administration (FDA) for vosoritide, an investigational medicine 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. Vosoritide has not been approved for use or determined to be safe or effective. BioMarin has submitted data that will now be reviewed by regulators who will decide whether to approve the drug to be marketed.

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 achondroplasia and the safety and efficacy of vosoritide. We are incredibly grateful to everyone who participates in our clinical studies.

Ongoing Study, Enrollment Open

111-209: Phase 2 Study for Children at Risk of Requiring Cervicomedullary Decompression Surgery

111-209 is open for enrollment, and the first participants have begun treatment.
The study is designed to study vosoritide's safety and efficacy in infants and children who are at risk of needing cervicomedullary decompression surgery.

For more information on 111-209, please visit:
https://clinicaltrials.gov/ct2/show/NCT04554940

Ongoing Studies, Enrollment Complete

111-202 and 111-205: Dose-Finding and Extension Studies

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

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

111-206 and 111-208: Phase 2 Safety and Efficacy Studies

Enrollment for 111-206 is now complete. After completing one year on this study, participants will be followed in the long-term extension study called 111-208. These clinical studies are designed to study vosoritide's safety and effect on participants' growth, need for surgeries, bone health, and quality of life.

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

111-301 and 111-302: Phase 3 Safety and Efficacy Studies

Enrollment for 111-301 is now complete, and BioMarin has announced primary endpoint results. Currently, participants are being followed in the long-term extension study called 111-302. Please see the "Publications and Presentations" section on page 3 for more details.

For more information on 111-301, please visit:
https://clinicaltrials.gov/ct2/show/NCT03197766

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

111-501 and 111-502: Observational Studies on Lifetime Impact

Enrollment for 111-501 (LIAISE) and 111-502 (LISA) are now complete. These studies do not involve an investigational medicine and aim to better understand what it is like to live with achondroplasia by finding health trends from childhood through adulthood. Please see the "Publications and Presentations" section on page 3 for more details.

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

111-901: An Observational Study

Enrollment for 111-901 is now complete. 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

Publications and Presentations

Endocrine Society (ENDO)

Combined results from BMN 111-301 and 111-302 were presented at the 2021 annual meeting of the Endocrine Society (ENDO) in March.

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

American College of Medical Genetics (ACMG)

Several presentations of data from BioMarin's clinical research program occurred at the American College of Medical Genetics (ACMG) annual meeting in April. These included 5-year data results from 111-202 and 111-205; a poster presentation of data from the global Achondroplasia Caregiver Survey; a poster of interim results from the Meaningful Outcomes market access survey; and an oral presentation and poster of results from BMN 111-501 (LIAISE), the observational study on lifetime impact.

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

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

For additional information:

- For information on BioMarin clinical studies, visit www.clinicaltrials.gov and type in the study code "BMN 111"
- For information and resources about living with achondroplasia, please visit www.achondroplasia.com
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at medinfoeu@bmrn.com