

# Update for Achondroplasia Associations

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

BioMarin has announced the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) 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 in Europe.

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 Studies, Enrollment Open

### 111-901: an observational study



111-901 is open for enrollment by invitation. 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. These clinical studies are designed to study vosoritide's safety and effect on participants' growth, need for surgeries, bone health and quality of life.

- Ages 0 to 5 years old, in three age groups (0 to 6 months, 6 months to 2 years, 2 to 5 years)
- Enrollment into the 111-206 study is currently open for newborns around 3 months old and younger
- For one year, half of the participants are on placebo (inactive substance) and half are on the investigational medicine
- During the first year, the study is blinded which means the participants and the doctors do not know whether the participant received a placebo or not
- After one year, all participants will receive the investigational medicine in the extension study BMN 111-208

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 closed for enrollment, and 111-502 (LISA) is open for enrollment until December 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 the phase 3 safety and efficacy study 111-301 is now complete, and BioMarin has announced primary endpoint results. Currently all participants are being followed in the long-term extension study called 111-302.



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 studies

BioMarin has completed the dose evaluation study 111-202 and is currently following all 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>

### For additional information on BioMarin achondroplasia clinical studies:



- Visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and type in the study code BMN 111
- For inquiries or to provide feedback from advocacy organisations, please contact [patientadvocacy@bmrn.com](mailto:patientadvocacy@bmrn.com)



- Contact BioMarin Medical Information at: [medinfoeu@bmrn.com](mailto:medinfoeu@bmrn.com)