

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 enrolment 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 enrolment 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.

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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 enrolment until the end of 2019, and 111-502 (LISA) is open for enrolment 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, Enrolment Complete



111-301 and 111-302: clinical studies

Enrolment 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>