

Newborn Infant Trial for Achondroplasia

BioMarin Pharmaceutical is investigating a new medicine, vosoritide or BMN 111, to treat newborn infants with achondroplasia. BMN 111 has not been approved for use or been determined to be safe or effective. It is only available as part of a clinical trial.

Clinical Trial Overview



Enrollment into the 111-206 study is currently open for newborns around 3 months old and younger.

Participants Must:

- join and participate in an observational study (111-901) for three months prior to receiving the study medicine or placebo (inactive substance); AND
- be screened and meet criteria in order to participate in the 111-206 study; AND
- live in or permanently relocate to a country conducting the 111-206 study (United States, United Kingdom, Australia or Japan)
- Globally, BioMarin is recruiting 20 participants into this age group within the 111-206 study
- For one year, half of the participants are on placebo and half are on the study medicine
- 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 be eligible to receive the study medicine in an extension study, 111-208. They will be eligible to continue in this clinical trial until they have reached near final adult height

Trial Participation



Clinical studies are designed to investigate the safety and efficacy of new medicines. A patient or caregiver's choice to participate in a study is an important personal decision. If you believe your patient meets these criteria and is interested in participating or wants to learn more about this study, please contact the research staff at the site closest to you or contact BioMarin Medical Information at MedInfo@bmrn.com. A full list of participating sites is listed below at our clinicaltrials.gov registry.

Study listing on: clinicaltrials.gov: https://clinicaltrials.gov/ct2/show/NCT03583697



Please note, participation in the clinical trial may have country specific limitations. For further information on BioMarin achondroplasia studies, please visit www.clinicaltrials.gov or contact BioMarin Medical Information at medinfo@bmrn.com.