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BIOMARIN ACHONDROPLASIA CLINICAL DEVELOPMENT PROGRAM An Update for Associations and Families

BioMarin Pharmaceutical Inc. is a global biotechnology company that has two decades of experience in addressing the complex needs of people, often children, with rare diseases. We aim to establish meaningful partnerships with advocacy groups, and the communities that support them. We know that participating in a clinical trial is filled with uncertainty and can be challenging. At BioMarin, we are focused on families and children affected by the conditions in which we work.

Currently, we have three ongoing clinical trials for achondroplasia. The first is a multinational observational trial (111-901) open from birth to 17 years of age. The ongoing observational trial is collecting consistent growth measurements for up to 7 years. This baseline information can then be compared to information from BioMarin's trial that includes an investigational therapy (both for individual children and for all children in total). The trial aims to enroll an equal number of boys and girls and has been ongoing since April of 2012; therefore, some children have been in the trial for several years. In this trial, participants do not receive the investigational therapy.

This trial is ongoing in many countries, including the US, Australia, France, Germany, Spain, Turkey and the UK. For more detailed eligibility criteria and current information about the sites, please visit: <https://clinicaltrials.gov/show/NCT01603095>

The second ongoing trial (111-205) is an open-label extension study for participants of our 111-202 trial that started in 2014 in the US, France, Australia and the UK. The term open-label means that both the clinical team and participant know which treatment is being administered. Approximately 30 participants, between the ages of 7 to 16 will receive one of three different dosing regimens daily. This trial will be fully enrolled by October 2017 and will last until 2022. For more information please visit: <https://clinicaltrials.gov/show/NCT02724228>

The third ongoing trial is our Phase 3, placebo-controlled trial (111-301). This trial is open to ages 5 to 17 years of age and includes approximately 110 participants. In this trial, participants have a randomized chance of receiving either placebo or BMN 111, BioMarin's investigational therapy for achondroplasia. The primary outcome of the trial is to evaluate change in the rate of bone growth. This trial lasts for 52 weeks. Trial participants will have to have completed a minimum of 6 months in the observational trial (111-901) before they can be screened for the Phase 3 (111-301) trial. Participants on placebo can roll over to treatment after a specified time. This trial started recently and more information can be found here: <https://clinicaltrials.gov/show/NCT03197766>



There is high interest in participation for the trials. Due to competitive enrollment across all of our trial sites globally, the availability of participation in the trials may be more limited in some countries than others. It is BioMarin's position that those eligible 111-901 participants who have been waiting the longest are prioritized into the 111-301 trial.

Please know that your voice is valued at BioMarin. For inquiries from advocacy organizations, please contact patientadvocacy@bmrn.com. For further information on BioMarin clinical studies, please visit www.clinicaltrials.gov or contact BioMarin Medical Information at medinfo@bmrn.com or by phone: Toll Free 1-800-983-4587.