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BioMarin Clinical Trial Programme Update for Hypochondroplasia

BioMarin is pleased to update the **Hypochondroplasia** community about our ongoing clinical development programme

We're aiming to understand the clinical course and growth of children with **hypochondroplasia**, through our

111-902 study

What Is the 111-902 Study?

The 111-902 study is an observational study to assess the clinical course and growth in children with hypochondroplasia.

Participation in the study involves collecting growth measurements and other information. This study does not involve any investigational treatments or medicine.

Many of the tests and procedures required for this study are also done as part of a child's normal care, such as having measurements and vital signs taken and having physical exams. For this study, there will be more measurements taken than usual to evaluate relevant scientific aspects of hypochondroplasia.

'111-902' denotes the study identifier number provided by BioMarin.

Study Duration

Participation in the 111-902 study lasts a minimum of 6 months and includes:

- Screening: Up to 35 days
- **Study visits:** Every 6 months until the child reaches final adult height (around age 16 years for girls or around age 18 for boys)

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To Join the study, the Child Must:

- Be age 15 years or younger
- Have been diagnosed with hypochondroplasia
- Be able to move and stand without assistance (does not apply to children under age 5 or those a bit over 3 feet tall)
- Have not received an investigational product or medical device within 6 months before the screening visit



Why Take Part?

- The 111-902 study will help us learn more about how children with hypochondroplasia grow and add significantly to the knowledge base, which could help others in the future
- In addition, a subgroup of eligible children from the 111-902 study will be asked to participate in a clinical trial of a new treatment for hypochondroplasia. To participate in the clinical trial, all children must first be enrolled in the 111-902 study for at least 6 months to assess their current growth



About Clinical Studies

- The 111-902 study is an observational clinical study. In an observational clinical study, participants agree to have information collected in this case growth information. In the 111-902 study, study doctors will analyze the data to learn more about hypochondroplasia, which may eventually help others in the future
- Clinical studies follow specific rules to protect the rights, safety, well-being, and confidentiality of study participants
- Participation in the 111-902 study is completely voluntary. Children participating in the study and their parents/guardians can end the child's participation at any time and for any reason without affecting their usual medical care

How Do I Get More Information? Contact BioMarin Medical Information at <u>medinfo@bmrn.com</u>
Visit ClinicalTrials.gov and search for Identifier: NCT06212947 or 111-902

About BioMarin

Since 1997, BioMarin has been dedicated to transforming lives through genetic discovery using a science-driven, people-forward approach. BioMarin develops targeted therapies that address the root cause of the genetic conditions they may treat, helping to better the lives of those living with rare genetic conditions – all through close collaboration with patients and patient advocates.