



UNIVERSITY
OF MANITOBA

Research Study of Children with Hypophosphatasia

Principal Investigator - Dr. Cheryl Rockman-Greenberg

What is the purpose of this study?

This study examines the safety of a drug called ENB-0040 and looks at the effects it has on children who have been diagnosed with hypophosphatasia (HPP) when given as an injection three times a week. This research is being done because currently, there is no effective treatment for HPP.

Who can participate?

Children aged 5-12 years of age who have been diagnosed with HPP.

What is involved?

- There are seven study visits (each approximately 4-6 weeks apart) in total. Total time of participation in the study will be approximately 7 months.
- Child will receive an injection of study drug under the skin, 3 times a week.
- Blood and urine samples
- X-rays of hands, wrists and knees
- DEXA – a standard x-ray test that measures the density of bones
- Dental X-rays
- Kidney Ultrasound
- Two Bone Biopsies – a pencil eraser-size piece of bone is removed from the hip bone and looked at under a microscope.
- Required to take a medication called Tetracycline prior to bone biopsies
- Physical therapist testing
- Two types of questionnaires

For more information on the study and to participate, contact Amy Yakimoski, Study Coordinator for Dr. Cheryl Rockman-Greenberg at ayakimoski@mich.ca