inozyme pharma

Introducing: The ENERGY 3 Study[1]

Investigating a potential treatment option for children with ENPP1 Deficiency (ARHR2)

Study design

- Multi-center, randomized in a 2:1 ratio, controlled, open-label Phase 3 study
- Will evaluate the efficacy and safety of INZ-701 in children with ENPP1 Deficiency
- INZ-701 is administered by subcutaneous injection

Select enrollment criteria

- Age ≥1 year to <13 years
- Confirmed postnatal molecular genetic diagnosis of ENPP1 Deficiency with biallelic mutations (ie, homozygous or compound heterozygous)
- Radiographic evidence of skeletal abnormalities at baseline

Study duration

- ≤52-day screening period (including a washout period of up to 7 days for prohibited medications post-randomization)
- 52-week randomized treatment period
- Follow-up open-label extension period

Learn more about the ENERGY 3 Study

Available Sites

Cook Children's Medical Center

Fort Worth, TX
Principal Investigator: Dr Joel Steelman
joel.steelman@cookchildrens.org

Children's Hospital of Philadelphia

Philadelphia, PA
Principal Investigator: Dr. David Weber
Contact: Max Krumpholz krumpholm1@chop.edu

Ann & Robert H. Lurie Children's Hospital

Chicago, IL
Principal Investigator: Dr. Jennie Miller
Contact: Madeleine Muller mmuller@luriechildrens.org

CHU Sainte-Justine

Montreal, Quebec
Principal Investigator: Dr. Nathalie Alos
Contact: Michna Alphonse kenny-michna.alphonse.hsj@ssss.gouv.qc.ca

Coming Soon

Nationwide Children's Hospital

Columbus, OH

Children's Hospital Colorado

Aurora, CO

Your pediatric patients with ENPP1 Deficiency may be eligible to participate.

To learn more, please contact a study site or Catherine Nester, Inozyme Pharma, at Catherine.Nester@Inozyme.com or call 717-587-0845.

Reference:
1. The ENERGY 3 study: evaluation of efficacy and safety of INZ-701 in children with ENPP1 deficiency. ClinicalTrials.gov identifier: NCT06046820. Updated September 21, 2023. Accessed September 22, 2023. https://clinicaltrials.gov/study/NCT06046820