



For more information about the ENPP1 Deficiency clinical trial, including how you can access our free genetic testing program, please visit InozymeStudies.com or email clinicaltrials@inozyme.com

An investigational therapeutic opportunity for patients with ENPP1 Deficiency (GACI or ARHR2)

ENPP1 Deficiency Clinical Trial

Enrollment is underway for patients with ENPP1 Deficiency (GACI or ARHR2) who are between the ages of 18 - 65. This multi-center, open-label, first in human study will assess the safety and tolerability of INZ-701, an investigational enzyme replacement therapy.

Key Eligibility Criteria

To be eligible to participate in this clinical trial, patients must meet the following criteria:

- Be male or female between the ages of 18-65 years of age at the time of screening
- Have a clinical diagnosis of ENPP1 deficiency supported by genetic testing
- PPI <1300 nM at screening

About ENPP1 Deficiency

ENPP1 Deficiency is a rare and life-threatening genetic disorder caused by mutations in the ENPP1 gene. People with ENPP1 Deficiency can have:

- hypophosphatemic rickets
- ligamentous calcification
- hearing loss
- joint pain
- A low amount of inorganic pyrophosphate (PPI)

Early symptoms can appear within the first few months of life as Generalized Calcification of Infancy (GACI) with high lethality rate.

About INZ-701

The study of INZ-701 is a first-in-human and first-in-patient, multiple ascending dose study followed by a long term extension period to evaluate safety and tolerability and identify the dose which restores PPI to therapeutically effective levels.

About Inozyme

Inozyme Pharma is a rare disease biopharmaceutical company developing novel therapeutics for treatment of diseases of abnormal mineralization impacting the vascular and soft tissue and skeleton.

Genetic Testing is Available

Consider getting your patients tested for ENPP1 Deficiency if they've experienced the symptoms described and do not have genetic confirmation of the disease. Individuals who meet the eligibility criteria can receive a no-cost genetic test through PreventionGenetics to determine if they have the condition or are a carrier for ENPP1 Deficiency.

Learn More or Refer a Patient

For more information on how to refer a patient to our clinical trial or about genetic testing options please visit

InozymeStudies.com or
email clinicaltrials@inozyme.com