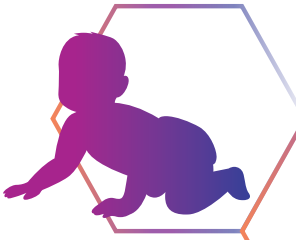
The ENERGY Study

Investigating BMN 401*, a subcutaneous ENPP1 enzyme therapy, for infants with GACI



Generalized Arterial Calcification of Infancy (GACI) is a rare, severe disease caused by mutations in the *ENPP1* (Type 1) or *ABCC6* (Type 2) genes that leads to high cardiovascular morbidity and mortality.[1]

There are currently no approved therapies for GACI.[2]

Study design[3]

- Phase 1b, open-label study (NCT05734196)
- To assess the safety, tolerability, pharmacokinetics, and pharmacodynamics of subcutaneous BMN 401 in infants with GACI

Select enrollment criteria[3]

- Birth (newborn) to <1 year of age at baseline
- Confirmed postnatal genetic diagnosis of ENPP1 or ABCC6 Deficiency with biallelic mutations (ie, homozygous or compound heterozygous)

Study duration[3]

*Formerly INZ-701. BMN 401 is an investigational product and has not been determined to be safe or effective by the US Food and Drug Administration or any other health regulatory authority

BIOMARIN®

Available Sites[3]

Children's Hospital of Philadelphia
Philadelphia, PA
Principal Investigator: Dr David Weber
weberd@chop.edu

Rady Children's Hospital
San Diego, CA
Principal Investigator: Dr Nathaly Sweeney
nsweeney@rchsd.org

Nationwide Children's Hospital
Columbus, OH
Principal Investigator: Dr Bimal Chaudhari
bimal.chaudhari@nationwidechildrens.org

Your patients with GACI may be eligible to participate.

References: 1. Ferreira CR, et al. *JBMR Plus.* 2025;9(5):ziaf019. **2.** Galletti S, et al. *JIMD Rep.* 2011;1:23–27. **3.** NCT05734196: https://clinicaltrials.gov/study/NCT05734196, accessed 06Feb2026.

BIOMARIN®