

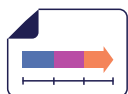
# The ENERGY Study

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.<sup>1</sup>

There are currently no approved therapies for GACI.<sup>2</sup>



## Study design<sup>3</sup>

- 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<sup>3</sup>

- 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<sup>3</sup>

≤60-day screening period	52-week treatment period	Follow-up extension period
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A large orange arrow pointing to the right, which contains the study duration information presented in the table above.

## Available Sites<sup>3</sup>



### 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. *JBM R 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.

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