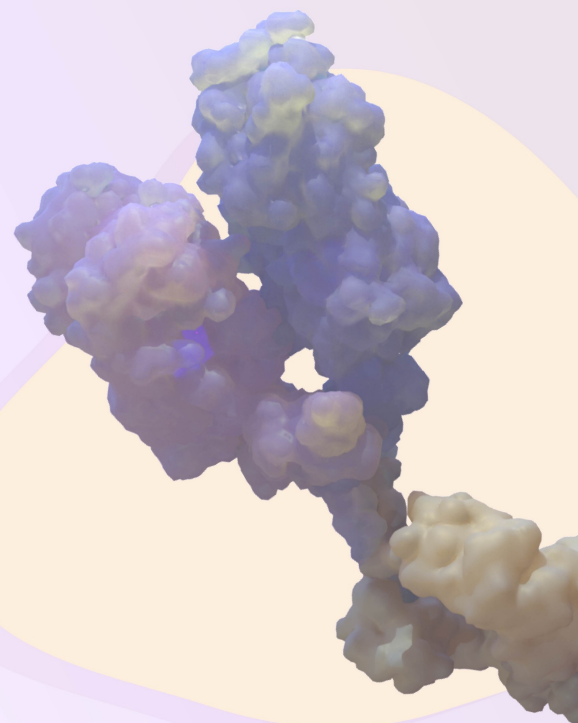


## Generalized Arterial Calcification of Infancy (GACI)

GACI is characterized by narrowing of large and medium arteries caused by severe and pathological vascular calcification and neointimal proliferation, resulting in dysfunction and potential failure of major organs, such as the heart, lungs and kidneys.



### Who Should Be Tested for GACI?

#### Indications to test

**Abnormal brightness**  
of arteries or heart valves (ultrasound, ECHO, CT, MRI)

OR

**Vascular stenosis**  
(CTA, MRA, ECHO)

OR

**Family history**  
of fetal/neonatal death or hypophosphatemic rickets



Genetic testing for **ENPP1** and **ABCC6**

ABCC6, adenosine triphosphate binding cassette subfamily C member 6; CT, computed tomography; CTA, computed tomography angiography; ECHO, echocardiogram; ENPP1, ectonucleotide pyrophosphatase/phosphodiesterase 1; MRA, magnetic resonance angiography; MRI, magnetic resonance imaging.  
**References:** 1. Mulcahy CH, et al. *J Congenit Cardiol.* 2019;3(1). doi:10.1186/s40949-018-0022-1 2. Nasrallah FK, et al. *Ultrasound Obstet Gynecol.* 2009;34(5):601-604. 3. Rutsch F, et al. *Circ Cardiovasc Genet.* 2008;1(2):133-140. 4. Ferreira CR, et al. In: Adam MP, et al. eds. *GeneReviews® [Internet].* University of Washington, Seattle; 1993-2020. 5. Ferreira CR, et al. *J Bone Miner Res.* 2021;36(11):2193-2202. 6. Ferreira CR, et al. *Genet Med.* 2021;23(2):396-407. 7. Chong CR, Hutchins GM. *Pediatr Dev Pathol.* 2008;11(5):405-415. 8. Ramirez-Suarez KI, et al. *Pediatr Radiol.* Published online April 19, 2022. doi:10.1007/s00247-022-05364-0

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To access the sponsored testing program, please reach out to Catherine Nester, Inozyme Pharma at [Catherine.nester@inozyme.com](mailto:Catherine.nester@inozyme.com) or 717-587-0845

# ENPP1 Deficiency Trial in Infants: The ENERGY-1 Study

## Study Overview

Study INZ701-104 (the ENERGY-1 study) is a Phase 1b, open-label study to assess the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of INZ-701 in infant subjects with ENPP1 Deficiency (Generalized Arterial Calcification of Infancy).

## Key Inclusion Criteria:

- Subject must have a post-natal confirmed molecular genetic diagnosis of ENPP1 Deficiency with biallelic mutations (ie, homozygous or compound heterozygous)
- Subject must be male or female ages birth to <1 year of age at Baseline
- Subject must weigh  $\geq 0.5$  kg at the time of the first dose of INZ-701

## Find a Study Site

### The Children's Hospital of Philadelphia

Philadelphia, Pennsylvania, United States, 19104

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### Boston Children's Hospital

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## Coming Soon

### University of Utah

Salt Lake City, Utah

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### Rady Children's Hospital

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<https://www.clinicaltrials.gov/study/NCT05734196>