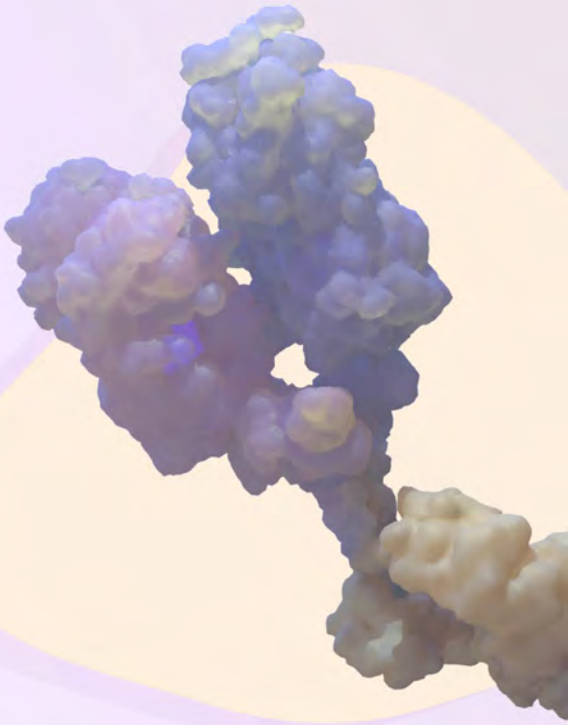
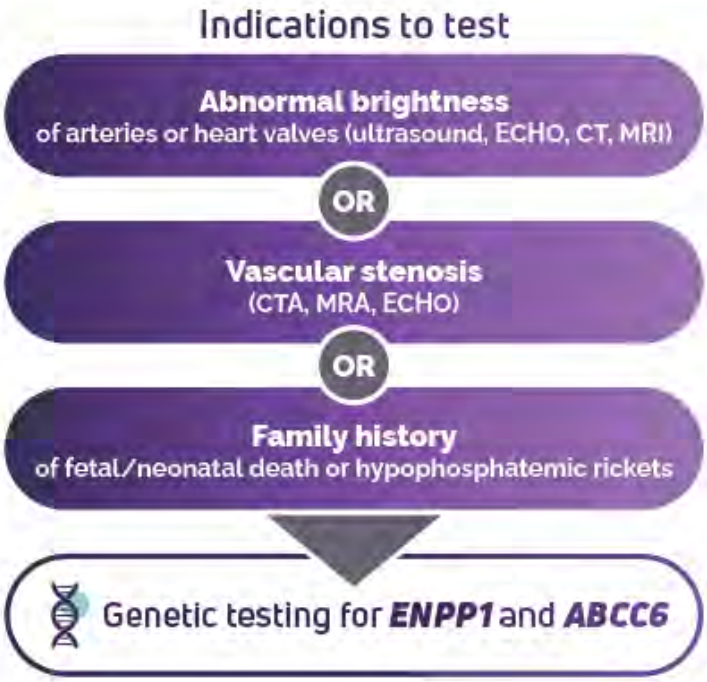
Inozyme Pharma is sponsoring ultra-rapid genetic testing at Rady Children's Institute for Genomic Medicine for infants suspected of having GACI

Generalized arterial calcification of infancy

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



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

To access the sponsored testing program, please reach out to Catherine Nester, Inozyme Pharma at 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 ≥ 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
Contact: Maximilian Krumpholz
267-432-0511
krumpholm1@chop.edu
Contact: Rachel Walega
267-586-5969
WALEGAR1@chop.edu

Boston Children's Hospital
Boston, Massachusetts, United States, 02115
Contact: Alayna Dutcher
617-355-0741
Alayna.dutcher@childrens.harvard.edu
Contact: Andrea Hale, RN, MHP
617-919-2867 andrea.hale@childrens.harvard.edu

Nationwide Children's Hospital
Columbus, Ohio
Contact: Bimal Chaudhari, MD
614-722-3535
Bimal.Chaudhari@nationwidechildrens.org
Contact: Marina Artemova, PhD
614-722-2655
Marina.artemova@nationwidechildrens.org

Rady Children's Hospital
San Diego, California
Contact: Phoebe Chandler
858-576-1700 x220096
pchandler@rchsd.org

University of Utah
Salt Lake City, Utah
Contact: Carrie Bailey
801-587-3605
Carrie.bailey@hsc.Utah.edu

Manchester Childrens Hospital
Manchester, UK
Contact: Charlotte Boe
charlotte.boe@mft.nhs.uk

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