The ENERGY Study: Evaluation of Safety and Tolerability of INZ-701 in Infants With ENPP1 Deficiency or ABCC6 Deficiency

NCT05734196

Status	RECRUITING
Phase	Phase 1
Sponsor	Inozyme Pharma
Enrollment	16 participants

Key Eligibility Criteria

Inclusion (5)

- Infant aged d 1 year at the time of enrollment
- Study participant must have a confirmed post-natal molecular genetic diagnosis of ENPP1 Deficiency or ABCC6 Deficiency
- Study participants must have clinical manifestations of generalized arterial calcification of infancy (GACI) or GACI-2, which must include at least one of the following: ectopic calcification, heart failure, respiratory distress, edema, cyanosis, hypertension, and cardiomegaly.
- Study participant must weigh e0.5 kg at the time of the first dose of INZ-701 in this study
- Written informed consent provided by a parent or legal guardian

Exclusion (5)

- In the opinion of the Investigator, presence of any clinically significant disease or laboratory abnormality that precludes study participation or may confound interpretation of study result
- Receiving end of life or hospice care
- Known malignancy
- Concurrent participation in another non-Inozyme interventional study
- Treatment with any non-Inozyme product or investigational device during study participation

Locations (7 total)

Rady Children's Hospital, San Diego, California, United States
Boston Children's Hospital, Boston, Massachusetts, United States
Nationwide Children's Hospital, Columbus, Ohio, United States
... and 4 more locations