

# A Real-world Long-term Safety and Immunogenicity Study of Olipudase Alfa Therapy in Pediatric Patients Less Than 2 Years of Age With Acid Sphingomyelinase Deficiency (ASMD)

NCT06192576

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Status	RECRUITING
Sponsor	Sanofi
Enrollment	10 participants

## Key Eligibility Criteria

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### Inclusion (5)

- The participant must have ASMD type A/B or B and must be <2 years of age at the time of treatment initiation, OR ASMD type A (without age restriction).
- The participant must weigh  $\geq 2$  kg [The United States Prescribing Information (USPI)] for olipudase alfa specifies this minimum weight for infants receiving olipudase alfa).
- The participant must have documented ASMD, as determined in peripheral leukocytes, cultured fibroblasts, or lymphocytes and/or by genotype determination.
- Signed informed consent must be provided by the participant's parent(s)/legal guardian(s), including compliance with the requirements and restrictions listed in the informed consent form (ICF) and in this protocol. The signed ICF must be provided before any protocol-related procedures are performed.
- The participant is eligible to start olipudase alfa enzyme replacement therapy or has received the first dose (and no more) of olipudase alfa, and has retrievable clinical, laboratory, and ADA data.

### Exclusion (3)

- The participant has received an investigational drug within 30 days or 5 drug half-lives before signature of the ICF and study enrollment.
- The participant is not suitable for participation for reasons determined by the Investigator, including medical or clinical conditions, or potential risk of noncompliance with study procedures.
- The participant is an immediate family member of employees of the study site or other individuals directly involved in study conduct, in conjunction with Section 1.61 of ICH-GCP Ordinance E6.

## Locations (5 total)

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Ann and Robert H Lurie Children's Hospital of Chicago- Site Number: 001002, Chicago, Illinois, United States  
Children's Hospital Medical Center- Site Number: 001003, Cincinnati, Ohio, United States  
Nationwide Children's Hospital - PIN- Site Number : 1001-1, Columbus, Ohio, United States  
... and 2 more locations

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<https://clinicaltrials.gov/study/NCT06192576>

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