

Analysis of Velmanase Alfa (Lamzedo®)'s Effects in the Body of Children With Alpha-Mannosidosis Under the Age 3

NCT06184503

Status RECRUITING
Sponsor Chiesi Farmaceutici S.p.A.
Enrollment 5 participants

Key Eligibility Criteria

Inclusion (6)

- Subjects with the provision of informed consent from their legal guardians (LAR)
- Have a confirmed diagnosis of alpha mannosidosis
- Have initiated treatment with velmanase alfa between birth to at least six weeks before turning 3 years of age
- Have information on the disease marker GlcNAc(Man)2 obtained:
 - before velmanase treatment initiation (ideally max 6 month before), and at least one post-treatment sample, collected following at least six weeks of treatment.
- ... and 1 more (see full listing online)

Exclusion (1)

- Participants who have undergone prior hematopoietic stem cell transplantation (HSCT) or other investigational therapies for treating alfa mannosidosis (supportive treatments acceptable).

Locations (2 total)

Nicklaus Children's Hospital, Miami, Florida, United States
Greenwood Genetic Center, Greenwood, South Carolina, United States