

Lysosomal Acid Lipase Deficiency in Risk Groups

NCT07455864

Status RECRUITING
Sponsor AstraZeneca
Enrollment 1,200 participants

Key Eligibility Criteria

Inclusion (21)

- Age 12 months to 18 years (infantile form is out of scope for the analytical component);
 - Patients not previously evaluated for LAL-D (test-naïve);
 - Presence of at least one (1) of the following major criteria:
 - Unexplained hepatomegaly and/or splenomegaly persisting e3 months;
 - Persistent hypertransaminasemia: ALT or AST e 1.5x upper limit of normal (ULN) after exclusion of common metabolic/infectious causes;
- ... and 16 more (see full listing online)

Exclusion (3)

- Confirmed alternative etiology fully explaining liver disease/dyslipidemia (e.g., hepatitis A/B/C, autoimmune hepatitis by diagnostic criteria) without grounds to suspect LAL-D;
- Wolman disease;
- Long-term use of systemic corticosteroids which is defined as oral or parenteral continuous administration during e14 days in the last 6 months prior to the inclusion.

Locations (1 total)

Research site, Nizhny Novgorod, Russia