A Multi-cohort Study of Safety, Efficacy, PK and PD of GNR-055 in Patients With Mucopolysaccharidosis Type II

NCT05208281

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	AO GENERIUM
Enrollment	32 participants

Key Eligibility Criteria

Inclusion (5)

- Signed inform consent;
- Verified diagnosis of MPS II (Hunter syndrome);
- Naïve patients or patients who have received standard ERT whit idursulfase products;
- No contraindications for lumbar puncture as judged by the Investigator;
- Willingness and ability to follow study procedures.

Exclusion (4)

- Clinically pronounced hypersensitivity to ID2S or any other component of the drug product;
- History of hematopoietic stem cell transplantation (HSCT) or bone marrow transplantation;
- Implanted or external non-removable metal devices, a cardiac pacemaker, or other objects sensitive to the magnetic field that may pose a danger to both the wearer and the correct operation of magnetic resonance imaging (MRI) equipment;
- Concomitant diseases and conditions that, in the Investigator's opinion, can put at risk the patient's safety during his/her participation in the study, or which will influence the safety data analysis in case of the disease/condition exacerbation during the study.

Locations (5 total)

Federal State-Funded Healthcare Institution Central Clinical Hospital of the Russian Academy of Sciences (Research Institute of Pediatrics and Child Health Protection of the Central Clinical Hospital of the Russian Academy of Sciences), Moscow, Russia

Federal State Budgetary Educational Institution of Higher Education "St. Petersburg State Pediatric Medical University" of the Ministry of Health of the Russian Federation, Saint Petersburg, Russia

V.I. Vernadsky Crimean Federal University, Simferopol, Russia

... and 2 more locations