

Effect of Lithium in Patients With Autism Spectrum Disorder and Phelan-McDermid Syndrome (SHANK3 Haploinsufficiency) (Lisphem)



Study Purpose

The primary purpose of the study is to evaluate the effect of lithium at 12 weeks (capsules dosed at 62.5mg, 125mg, and 250mg), compared to placebo, on social communication deficit in patients with Phelan-McDermid syndrome. The secondary objectives of the study are to evaluate the effect of Li+ at 12 weeks on all main symptoms in patients suffering from Phelan-McDermid Syndrome (PMS) including evaluating the tolerance of Li+ for 12 weeks, residual effects after stopping treatment, and parents' feelings at the end of the study regarding the child's behavior and impact on their daily life.

From Dr. Thomas Bourgeron: We have previously shown that lithium can restore synaptic dysfunction caused by a defect in the *SHANK3* gene.

The treatment of the study is lithium carbonate: Li+ carbonate are in capsule form. Inclusion will be ensured by the clinical genetics centers. Psychiatric evaluations will be carried out by the investigative child psychiatry service. Patients will be followed up by a child psychiatrist and a physician from the clinical investigation center.

Recruitment Criteria

Study Type: Interventional

Potential participants must:

- 7 Years to 18 Years
- Minimum weight of 20 kg
- Have a diagnosis of Phelan-McDermid syndrome
- Autism Spectrum Disorder
- Non-participation in another clinical trial

Trial Details

ClinicalTrials.gov ID: NCT04623398

Phase: 3

Duration: 28 - 30 weeks

Number of estimated participants enrolled: 22

Lead Sponsor: Assistance Publique - Hôpitaux de Paris

Countries: France

Trial Sites

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Questions

More information on [the study](#) can be found [here](#) and [here](#). For additional recruitment criteria, visit clinicaltrials.gov. To discuss enrollment and questions about recruitment, please reach out to the site coordinator listed above.

Additional Links:

- <https://www.aphp.fr/registre-des-essais-cliniques/effet-du-lithium-chez-les-patients-ayant-un-trouble-du-spectre>
- [Eligibility Criteria](#)
- [Clinical Trial Recruitment Announcement](#)
- [Understanding Clinical Research](#)
- [Clinical Trial Informed Decision Tool](#)
- [Clinical Trials in PMS Conference Session](#)