

IntraBio Ltd. is pleased to confirm the Phase III pivotal trial with N-acetyl-L-leucine (IB1001-301) is active and recruiting in the United States at the Mayo Clinic, MN.

The study, which investigates N-acetyl-L-leucine (IB1001) for the treatment of Niemann-Pick disease Type C (NPC), will enroll a total of approximately 52 patients aged 4 years and older across all international sites.

Recruitment is expected to be completed by December 2022.

To ensure all patients have the opportunity to participate, interested patients are encouraged to contact Mayo as soon as possible to schedule a screening visit before enrollment is complete. In the United Kingdom, three trial sites have been established. Information on each site, including contact details, can be found below.

The Mayo Clinic

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IB1001 OVERVIEW

IB1001-301 is a multinational, randomized, placebo-controlled, double-blinded, crossover Phase III study. Patients will receive treatment with both IB1001 orally administered sachet) and a matching Placebo in two, 12-week treatment periods. Patients who complete the study will have the option to participate in an open-label extension phase, where patients will receive treatment with IB1001 for a minimum of 1-year.

Patients aged 4 years + may be eligible for recruitment at all trial sites. Patients are required to have neurological symptoms and cannot be using any other investigational agent (including investigational drugs in expanded access programs). Patients are permitted to use a stable dose of miglustat.

For the complete enrolment criteria, as well as details regarding the study assessments, multinational clinical trial sites, etc., please visit ClinicalTrials.Gov ([NCT05163288](https://clinicaltrials.gov/ct2/show/study/NCT05163288)).

If you have any additional questions on the study/recruitment, please contact:

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