IntraBio Inc. is pleased to announce the commencement of its Pivotal Trial: IB1001-301, "Effects of N-Acetyl-L-Leucine on Niemann-Pick disease type C (NPC): A Phase III, randomized, placebo-controlled, double-blind, crossover study."

The trial will be conducted at 16 multinational sites in Australia, Europe, the United Kingdom, and United States.

In the United Kingdom, there will be 4 trial sites which are planned to open for recruitment in July 2022. Given the level of interest in the study, it is expected that the trial will enrol very quickly, and that recruitment will be completed by December 2022. Patients and families who are interested in learning more about the trial, including the eligibility criteria and enrolment process, are therefore encouraged to contact their prospective study site.

**Great Ormond Street Hospital**
Trial Site for Patients Aged 4 – 17 Years
PI: Paul Gissen, MD
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**Royal Free London NHS Foundation Trust**
Trial Site for Patients Aged 13 Years +
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**Royal Manchester Children's Hospital**
Trial Site for Patients Aged 4 – 17 Years
PI: Simon Jones, MD
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Trial Site for Patients Aged 18 Years +
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## IB1001-301 OVERVIEW

A brief summary of the eligibility criteria, trial design, and trial sites is provided below. For the complete enrolment criteria, as well as details regarding the study assessments, multinational clinical trial sites, etc., please visit ClinicalTrials.Gov (NCT05163288).

### Study Drugs

N-acetyl-L-leucine (IB1001) and the matching Placebo are orally administered as sachets which are mixed with water, orange juice, or almond milk. They are compatible with feeding tubes.

### Eligibility Criteria

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.

### IB1001-301 Study Design

IB1001-301 is a multinational, randomized, placebo-controlled, double-blinded, crossover Phase III study. Patients will receive treatment with both IB1001 and a matching Placebo over the course of the study.

Patients will be assessed at 6 study visits during three study periods: a baseline period (approximately 14 -21 days), the first intervention period ("Period I"; approximately 84 -91 days), and the second intervention period ("Period II"; approximately 84 -91 days). Patients will be assessed twice during each intervention period **(Figure 1).**

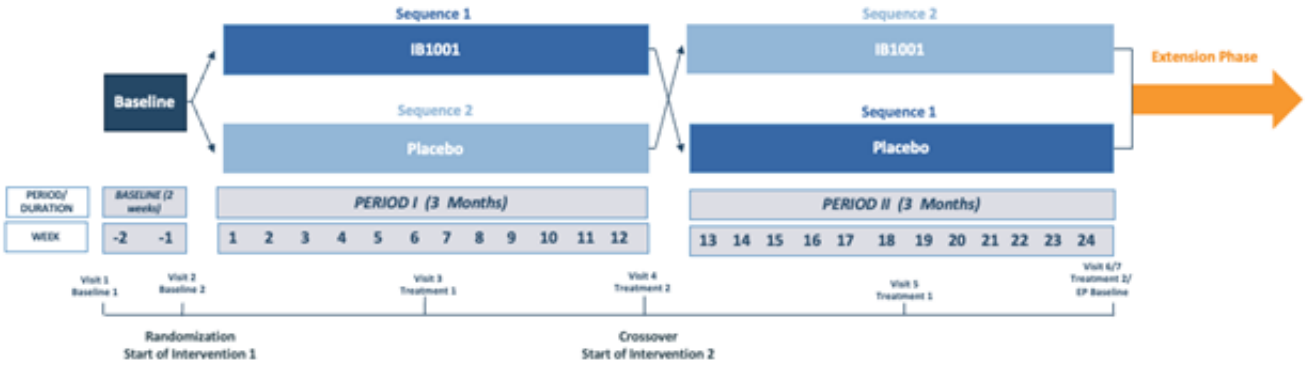


**Figure 1: Study Schema for Naïve patients.**

***Prohibited Medications:*** Patients using Tanganil (or other forms of N-acetyl-leucine not provided in IB1001-301) will be required to stop taking the medicine ("washout") for 6-weeks prior to screening (Visit 1) and must remain off Tanganil /other forms of N-acetyl-leucine throughout the duration of the trial.

***Randomization:*** At **Visit 2** (Baseline 2), patients will be randomly assigned (1:1) to two randomization sequences:

- **Sequence 1:** Starting **Visit 2 (**Baseline 2), patients will receive IB1001 during Period I (for approximately 84 days). At the end of Period I (Visit 4), the patient will "crossover" and immediately crossover receive placebo during Period II (for approximately 84 days).
- **Sequence 2:** Starting **Visit 2** (Baseline 2), patients will receive placebo during Period I ((for approximately 84 days). At the end of Period I (Visit 4), patients will "crossover" and immediately receive IB1001 during Period II (for approximately 84 days).

***Treatment:*** During the trial, every patient will receive approximately 12-weeks of treatment with IB1001, and 12-weeks of placebo. Patients, their family, and the study team will not know when they are on treatment with IB1001 or placebo.

***Study Assessments:*** Standard functional assessments (e.g., SARA, SCAFI, NPC-CSS, mDRS), will be performed, as well as quality of life questionnaires. There are no invasive procedures (i.e., lumbar punctures). Blood and urine samples will be collected, and physical examinations and 12-lead ECGs will be performed.

## Extension Phase

Patients who complete the study (Visit 6) can (if their Principal Investigator (PI) determines it is in their best interest) participate in an open-label extension phase, where patients will receive treatment with IB1001 for a minimum of 1-year.

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

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