

IB1001-202 CLINICAL TRIAL

Effects of N-Acetyl-L-Leucine on GM2
Gangliosidosis (Tay-Sachs and Sandhoff Disease):
A multinational, multicenter, open-label,
rater-blinded Phase II study

Information for European Clinical Trial Centres

Study IB1001-202 is a multinational, open-label, rater-blinded Phase II study that investigates **IB1001** for the treatment of **GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease)**. GM2 Gangliosidosis is a rare (“orphan”), inherited lysosomal storage disorder that results in progressive neurodegeneration, and for which there are currently no medically available treatments.

What is the purpose of study IB1001-202?

- The goal of IB1001-202 is to demonstrate that IB1001 is effective at improving the symptoms, functioning and quality of life in patients with GM2 Gangliosidosis after six (6) weeks of treatment
- The study also aims to demonstrate that IB1001 is safe and well-tolerated

What is IB1001?

- IB1001 is a modified amino acid, N-Acetyl-L-Leucine, that is orally administered (swallowed as a drink)
- N-Acetyl-L-Leucine has previously been investigated using a different formulation (same compound administered in a different way) in 192 patients with a vertigo disorder and 67 healthy volunteers
- The information collected to date shows that the drug is well tolerated and has a good safety profile

What should patients and their caregivers know about the study?

- The study will compare patients' symptoms before they start treatment (“baseline”), over a six (6) week treatment period with IB1001, and over a six (6) week period where patients stop taking IB1001 (“washout period”)
- There is no placebo arm of the trial. All patients enrolled in the study will receive treatment with IB1001
- After the end of the trial, an optional rollover study is planned, where patients would receive treatment with IB1001 for 1 year

Who is eligible to participate in the trial?

The Principal Investigator at each clinical site is ultimately responsible for determining if a patient is eligible to participate in the clinical trial. European patients may be eligible to participate if they are:

- Aged 6 years and older
- Have a confirmed genetic diagnosis of GM2 Gangliosidosis (Tay-Sachs or Sandhoff Disease)
- Are not using, or agree to stop using, medications which are not allowed (“prohibited” medications, e.g. Tanganil®) for six (6) weeks prior to beginning the assessment visits, and throughout the duration of the study
- The complete enrollment criteria can be found at [ClinicalTrials.gov \(NCT03759665\)](#)

How long is the study?

- Patients will take part in the study for approximately 3-4 months
- There are a total of 6 visits to the study site throughout the trial
- If, at their first visit to the site, patients are using or have used any medication that is not allowed (a “prohibited medication”) within the past six (6) weeks, there will be 1 additional site visit, for a total of 7 visits to the study site

What assessments are made in the study?

- Patients will be asked to perform standardized assessments that measure symptoms of GM2, such as ataxia. These assessments will include validated functional scales like the Scale for the Assessment and Rating of Ataxia (SARA) and Spinocerebellar Ataxia Functional Index (SCAFI)
- Patients and their family/caregiver will be asked to complete questionnaires about quality of life
- Video recordings will be made of patients walking and using their hands
- No invasive biomarkers will be assessed. Only limited blood draws and urine samples will be taken for routine clinical safety laboratory tests, and sparse PK sampling

What are the pros and cons of participating in the study?

The study is confidential

- Patient information and participation is confidential. No reports will contain a patient's name or personal information

The study is free

- Patients will receive reimbursement for some expenses related to participating in this study, such as travel or parking

The study contributes to the scientific understanding of GM2 Gangliosidosis

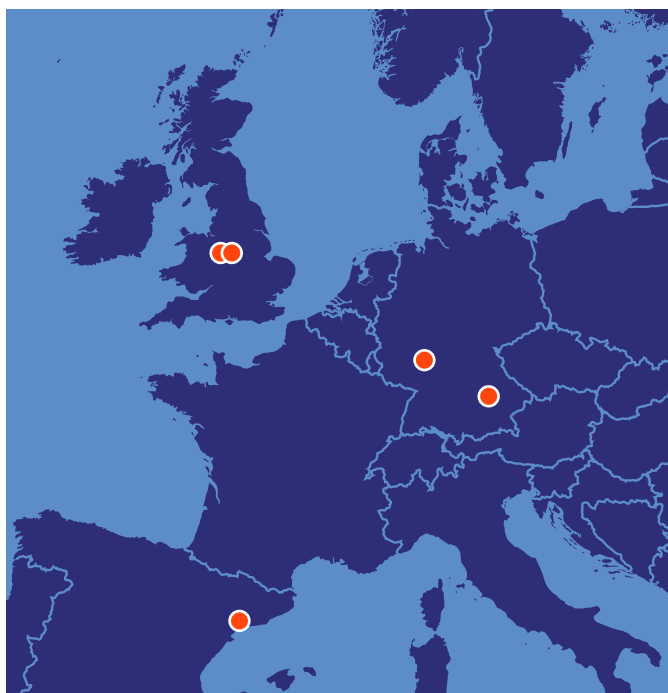
- Participation in the trial contributes to the scientific understanding of GM2 and may help treat patients with GM2 Gangliosidosis. However, patients who participate in the study may not have a clear benefit themselves

The study is optional

- Taking part in the study is entirely voluntary
- Patients can decide to stop participating in the study at any time, and their normal medical care will not be affected

The study can be associated with risks

- Any clinical study can be risky, and there may be risks associated with taking part in this study
- The study doctor can explain the specific risks associated with this study, and potential alternatives. It is important that patients, their families, or caregivers ask any questions they have



Planned clinical trial sites

Please see the map and the list below:

SPAIN

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**For additional information about IB1001-202,
please visit [ClinicalTrials.Gov \(NCT03759665\)](https://clinicaltrials.gov/ct2/show/study/NCT03759665)**

For additional information about GM2 Gangliosidosis

