Therapeutic Update

Pivotal Clinical Trial

Based on the robust evidence from the IB1001-201 Phase II clinical trial (NCT03759639) demonstrating IB1001's positive effect on symptoms, functioning, and quality of life for pediatric and adult patients with NPC (Bremova et al. 2021), IntraBio is initiating a Phase III pivotal trial with IB1001 for NPC (Clinical IB1001-301).

The trial is a 24-week randomized, double-blinded, placebo-controlled, crossover study that will enroll patients 4 years and older at approximately 16 trial sites in the United States, Europe, and Australia.

The full study procedures and eligibility criteria are posted on ClinicalTrials.Gov (NCT05163288).

Eligibility Criteria

Patients aged 4 years + may be eligible for recruitment at sites in Australia, Europe, and the United States. 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 (Zavesca®).

Study Design

During the trial, all patients will receive treatment with both IB1001 and placebo during two different "treatment periods," each lasting approximately 12-weeks each. The total duration of the study (with the screening period) will be approximately 26 weeks, during which there will be 6 study visits to the trial site.

Patients who complete the study will be eligible to join an open-label extension phase, where they will receive treatment with IB1001.

Additional Information

Trial sites are currently planned in Australia, the Czech Republic, Germany, the Netherlands, Slovakia, Switzerland, the United Kingdom, and the United States.

IntraBio will cover travel expenses (e.g. transport, hotel, food, COVID-19 tests) for the patient + 1 family member/caregiver to attend each study visit.

Recruitment

IntraBio plans to be open for recruitment in all countries in Q2 2022.

For further information on the IB1001-301 clinical trial or questions contact:

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