IntraBio Inc is pleased to announce that ***The New England Journal of Medicine*** (NEJM) has published the detailed results of the Phase 3, Pivotal Trial with N-acetyl-L-leucine (IB1001) for the treatment of Niemann-Pick disease Type C (NPC).

**New England Journal of Medicine: Trial of N-Acetyl-l-Leucine in Niemann–Pick Disease Type C**

The publication presents the efficacy and safety data from the Pivotal, randomized, placebo-controlled, crossover trial (IB1001-301; NCT05163288), where IB1001 showed a statistically significant improvement in neurological signs and symptoms, functioning, and quality of life in pediatric and adult NPC patients with IB1001 versus placebo. As previously reported, the trial met its primary and secondary endpoints and showed a very favourable safety profile with no serious adverse reactions.

A short Quick Take, an animated two-minute video, summarizing the highlights and key findings from the research article, is also available on **NEJM.org**

"The publication of the IB1001-301 clinical trial results by the New England Journal of Medicine reinforces our findings that IB1001 is a breakthrough for the treatment of Niemann-Pick disease type C and neurodegenerative diseases" said Kyriakos Martakis, MD, Principal Investigator from Justus Liebig University of Giessen, Germany. "The findings of this placebo-controlled Phase III trial match what we saw in the Phase IIb trial, where IB1001 also improved patients' symptoms and significantly improved their everyday functioning (e.g., in motor and cognitive performance) and quality of life. In the extension phase of this study, we saw long-term treatment had a clear disease-modifying effect. It is my hope that IB1001 is approved as quickly as possible so it can be available for NPC patients, as I strongly believe IB1001 will change the course of this disease."

IntraBio has filed a New Drug Application (NDA) with IB1001 for the treatment of NPC with the US Food and Drug Administration (FDA) in January 2024. The FDA has a 60-day filing review period to determine whether the NDA is complete and accepted for review. IntraBio intends to submit its Marketing Authorization Application to the European Medicines Agency in Q2 2024. IntraBio remains dedicated to making IB1001 available to patients and families as quickly as possible to help meet the NPC community's urgent unmet medical needs.