IntraBio Announces Positive Pivotal Trial Results of IB1001 for the Treatment of Niemann-Pick Disease Type C

- Phase 3 trial met the primary endpoint and key secondary endpoints showing high statistical significance

- IB1001 showed a clinically meaningful improvement in symptoms, functioning, quality of life, and cognition in both pediatric and adult patients with NPC

- IB1001 was safe and well-tolerated with a favorable safety profile consistent with previous clinical and pre-clinical studies

- Based on these positive results, IntraBio plans to file for marketing authorization with IB1001 with the FDA and EMA

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IntraBio Inc today announced positive topline results from the pivotal, Phase III IB1001-301 clinical trial (NCT05163288), showing IB1001 significantly improved symptoms, functioning, quality of life, and cognition in pediatric and adult patients with Niemann-Pick disease Type C (NPC).

The primary endpoint of the trial evaluated the impact of IB1001 on the Scale for the Assessment and Rating of Ataxia (SARA) compared to placebo after 12 weeks. Treatment with IB1001 demonstrated a statistically significant and clinically meaningful 1.37-point reduction of the SARA score compared to placebo (-1.97 on IB1001 vs. -0.60 on placebo; $p<0.001$). The trial also met its secondary endpoints, the modified Scale for the Assessment and Rating of Ataxia (mSARA) (-1.66 on IB1001 vs. on -0.67 placebo; $p<0.001$) and the Investigator's Clinical Global Impression of Change (CGI-C) (-0.7 on IB1001 vs. +0.1 on placebo; $p<0.001$). IB1001 was observed to be safe and well-tolerated, with no drug-related serious adverse events, consistent with its established, benign safety profile.

The positive IB1001-301 results are consistent with the Phase IIb multinational clinical trial previously completed with IB1001 for NPC (IB1001-201 - NCT03759639), which also showed a statistically significant and clinically meaningful improvement on the SARA, mSARA, and Investigator's CGI-I endpoints and a benign safety profile. Based on these positive trials, IntraBio will proceed with global regulatory submissions to the United States Food and Drug Administration (FDA), the European Medicines Agency (EMA), and other global regulators.

"This is a critical breakthrough for the NPC patient community," said Dr. Marc Patterson, Professor of Neurology, Pediatrics, and Medical Genetics at Mayo Clinic. "Conducting clinical trials in ultra rare, clinically variable disorders such as Niemann-Pick disease, type C, is extremely challenging, owing to the limited pool of potential participants and difficulty in demonstrating an effect in patients with existing neurological impairment. The trial of IB1001 shows clinically meaningful effects at a high level of statistical significance in patients with NPC. There is currently no approved therapy for NPC in the US, and given this convincing evidence of efficacy coupled with the very benign safety profile, I am hopeful that IB1001 will be rapidly approved, to ensure that every NPC patient will be able to access safe and effective treatment for this devastating disease."

The National Niemann-Pick Disease Foundation's Executive Director Joslyn Crowe commented: "We are optimistic IB1001 is quickly on track to be an approved treatment for NPC. The strong efficacy and safety results of the IB1001 clinical trial bring new levels of hope to patients and families affected by NPC. We look forward to continuing to collaborate with IntraBio to help ensure IB1001 is made available for all patients in our community given their urgent need for effective, approved treatments."

About IB1001

IB1001 is a novel, orally administered therapy with a unique mechanism of action offering neuroprotective and symptomatic benefits for rare and common neurological disorders. IB1001 is initially being developed as a potential treatment for rare lysosomal storage disorders, including NPC, GM1 and GM2 gangliosides, and inherited cerebellar ataxias.

IB1001 has been granted 13 Orphan Drug Designations from the FDA and EMA (for the treatment of NPC, GM1 gangliosides, GM2 gangliosides (GM2), Spinocerebellar Ataxias - 40+ subtypes, Ataxia-Telangiectasia (A-T), and Ataxia Ocular Motor Apraxia type 4) and three Rare Pediatric Disease Designations (RPDD) from the US FDA for the treatment of NPC, GM2, and A-T. IB1001 has also received Fast Track designation from the US FDA for NPC and GM2.

About IB1001-301

IB1001-301 (NCT05163288) is a multinational, randomized, placebo-controlled, crossover trial that evaluates the safety and efficacy of IB1001 in pediatric and adult patients with NPC. Patients aged 4 years and older were screened at trial sites in Australia, Europe, the United Kingdom, and the United States.

Patients were assessed during a baseline period and then randomly assigned (1:1) to receive orally administered IB1001 or placebo for 12 weeks. At the end of the 12-week treatment period, patients crossed over and initiated therapy with the alternate study drug (IB1001 or placebo) over the subsequent 12-week period. Patients who completed the study had the option to participate in an open-label Extension Phase.

About IntraBio

IntraBio Inc, a US biopharmaceutical company, is focused on the development of novel drugs addressing rare and common neurological diseases. IntraBio's platform technologies result from decades of research and investment at premier universities and institutions worldwide. Its clinical programs leverage the expertise in lysosomal function and intracellular signaling of its scientific founders from the University of Oxford and the University of Munich.

IntraBio's management team and scientific founders have a successful track record of drug development and commercialization in the USA and Europe. IntraBio's team translates innovative scientific research in the fields of lysosomal biology, autophagy, and neurology into novel drugs for a broad spectrum of genetic and neurodegenerative diseases so to significantly improve the lives of patients and their families.