IntraBio Reports Further Detail on Positive Data from IB1001 Multinational Clinical Trial for the Treatment of Niemann-Pick disease Type C

- IB1001 demonstrated statistically significant and clinically meaningful change in both the primary and secondary endpoints
- Significant improvement in gait, fine motor skills, speech, cognition, overall functioning and quality of life reported
- IB1001 rapidly improved both motor and cognitive symptoms in 6-weeks, consistent with its pharmacological action
- Subgroup analysis across the endpoints demonstrate consistent clinical effects across all demographics
- IB1001 was safe and well-tolerated with no serious adverse reactions
- IntraBio has initiated filing preparations and is continuing to engage with regulatory agencies on an approval path

Oxford, U.K. -- IntraBio Inc today announced positive results from the full data set for its multinational clinical trial of IB1001 (N-acetyl-L-leucine) for the treatment of Niemann-Pick disease Type C (NPC). In total, 33 subjects aged 7 to 64 years with a confirmed diagnosis of NPC were enrolled across 9 clinical trial sites in the United States, United Kingdom and Europe.

Key Findings

Efficacy

A total of 33 patients were recruited into the study and 32 patients were included in the mITT analysis set. IB1001 demonstrated a statistically significant and clear clinically meaningful improvement in symptoms, functioning, and quality of life for pediatric and adult patients with NPC. Treatment with IB1001 resulted in a statistically significant change in the Clinical Impression of Change in Severity (CI-CS) (90% CI: 0.25, 1.75, p-value = 0.029) assessed by blinded, independent raters.

The trial also met its secondary endpoints, including the Scale for the Assessment and Rating of Ataxia (SARA), Investigator's Clinical Global Impression of Change (CGI-C) demonstrating both a statistically significant change during treatment (clinical improvement; SARA 90% CI: -1.8, -0.5, p=0.001; CGI-C 90% CI: 3.0, 3.5, p <0.001) and during post-treatment washout (clinical deterioration; SARA 90% CI: 0.5, 2.0, p=0.002; CGI-C 90% CI: 4.0, 5.0, p=0.006). There was no meaningful difference on these endpoints detected between the baseline and washout period, bolstering the statistical significance and clinically meaningful effect of IB1001 established by the primary and secondary endpoints.

Subgroup analysis of the primary and secondary endpoints demonstrate consistent clinical effects across all demographics (age, gender, disease severity, age of symptom onset, etc.). This positive data provides a strong rationale for IB1001 to be used in the treatment of all patients with NPC.

Safety

IB1001 was observed to be safe and well-tolerated, with no drug-related serious adverse events.

Next Steps for NPC Clinical Study - IB1001-201

An Extension Phase with IB1001 is ongoing to confirm the treatment's long-term neuroprotective effects. The rational for IB1001's disease-modifying effect is strongly supported by earlier non-clinical studies in the NPC mouse model and results from long-term clinical case-series for NPC. IntraBio understands the urgency of making IB1001 available to NPC patients as soon as possible and will continue to engage with regulatory agencies with respect to an accelerated approval path.

Professor Susanne Schneider, MD, Principal Investigator, Ludwig Maximilians University of Munich said: "This treatment is a breakthrough for the NPC patient community. The statistically significant and clinically meaningful response in primary and topline secondary endpoints with IB1001, together with its compelling safety profile, easy oral administration [sachet mixed with water] affirm the very favorable risk/benefit profile of IB1001 as a treatment for this devastating disease."

The National Niemann-Pick Disease Foundation's Executive Director Joslyn Crowe commented: "The exciting results of the IB1001 clinical trial bring new levels of hope and optimism to patients and families affected by Niemann-Pick disease type C. There continues to be a critical need for new therapies to treat NPC and we are so grateful to our partners like IntraBio for their continued commitment to finding new treatments like IB1001."

Next Steps for IntraBio

In addition to Clinical Study IB1001-201, IntraBio is completing parallel multinational clinical trials with IB1001 for the treatment of GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease; NCT03759665) and Ataxia-Telangiectasia (A-T; NCT03759678). The IB1000 series has demonstrated efficacy (non-clinical and/or clinical) for a broad series of rare, common, and acquired neurological disorders including Alzheimer's Traumatic Brain Injury, and Dementias, which IntraBio is preparing to initiate clinical trials for with IB1001.

Mallory Factor, Chairman, said: "IB1001 is part of IntraBio's broad platform of novel treatments to provide neuroprotection, disease modification and symptomatic relief from multiple neurodegenerative and lysosomal storage diseases. In addition to our immediate priority of making IB1001 available for patients with NPC, GM2 Gangliosidosis and Ataxia-Telangiectasia, we will continue to investigate the neuroprotective, disease modifying, and symptomatic treatment effects for other neurological disorders with unmet medical needs."

About Niemann-Pick Disease Type C

Niemann-Pick disease Type C (NPC) is a rare (1:100,000 live births), prematurely fatal, autosomal recessive, lysosomal storage disorder. The disease begins in early childhood and presents with systemic, psychiatric, and neurological symptoms, including cerebellar ataxia. NPC is chronic and progressive in nature and is characterized by rapid degeneration of the cerebellum and major organ systems which severely impacts the quality of life. There is no approved treatment for NPC in the United States. Treatment of NPC in the European Union and several other countries is limited to substrate reduction therapy drug miglustat (Zavesca™).

About IB1001-201 Trial

IB1001-201 (NCT03759639) is a multinational clinical trial evaluating IB1001 for both the symptomatic and neuroprotective, disease-modifying treatment for adult and pediatric patients with NPC. Patients aged 6 years and older were enrolled at trial sites in the United States, the United Kingdom, the European Union.

To investigate both the symptomatic and neuroprotective, disease-modifying effects of treatment, IB1001 is assessed during two treatment sequences: a "Parent Study" consisting of a baseline period (with or without a study-run in), a 6-week treatment period, followed by a 6-week post-treatment washout period as an internal control for examining symptomatic relief, and an "Extension Phase", during which patients receive treatment with IB1001 for 1 year to study the neuroprotective, disease-modifying effects. Both the symptomatic and long-term benefits of treatment have previously been observed in observational clinical studies and are consistent with the pharmacological action of IB1001 demonstrated in in vitro and in vivo non-clinical studies.

About IntraBio

IntraBio Inc is a biopharmaceutical company with a late-stage drug pipeline including novel treatments for common and rare neurodegenerative 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 calcium signaling of its scientific founders from the University of Oxford and the University of Munich.

IntraBio's management team and consultants have a successful track record of drug development 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.

IntraBio Inc is a US corporation with its principal operations in Oxford, United Kingdom.

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