- ***First positive clinical trial for GM2 Gangliosidosis – 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 related serious adverse reactions***

OXFORD, UK / ACCESSWIRE / September 14, 2021 / 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 GM2 Gangliosidosis ("GM2"; Tay-Sachs and Sandhoff disease). In total, 30 subjects aged 6 to 55 years with a confirmed genetic diagnosis of GM2 were enrolled across 8 clinical trial sites in the United States, United Kingdom and Europe.

Key Findings

Efficacy

A total of 30 patients were recruited into the study and 29 patients were included in the primary modified intention-to-treat (mITT) analysis set. IB1001 demonstrated a statistically significant and clinically meaningful improvement in symptoms, functioning, and quality of life for pediatric and adult patients with GM2 Gangliosidosis. Treatment with IB1001 resulted in a statistically significant change in the Clinical Impression of Change in Severity (CI-CS) (90% CI: 0.00, 1.50, p-value = 0.039) assessed by blinded, independent raters (professors of neurology with expertise in movement and neurological disorders).

The trial also met its secondary endpoints, including the Scale for the Assessment and Rating of Ataxia (SARA), the Investigator's Clinical Global Impression of Change (CGI-C), and the Modified Disability Rating Scale (mDRS), demonstrating a statistically significant, clinical improvement with IB1001 treatment (SARA 90% CI: -1.75, -0.75, $p<0.001$; CGI-C 90% CI: -1.0, -0.5 $p<0.001$; mDRS 90% CI: -1.5, 0.0, $p=0.02$) and a statistically significant, clinical deterioration during post-treatment washout from IB1001 (SARA 90% CI: 0.5, 2.0, $p<0.001$; CGI-C 90% CI: 0.5, 1.0 $p<0.001$; mDRS 90% CI: 0.5, 1.5, $p<0.001$)).

Subgroup analysis of the 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 GM2 Gangliosidosis.

Safety

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

Summary

The positive results of this IB1001-202 study are reinforced by the efficacy and safety profile of IB1001 already demonstrated in IntraBio's successful IB1001-201 study for Niemann-Pick disease Type C (NPC). As is the case in the IB1001-202 clinical trial for GM2 Gangliosidosis, IB1001-201 was the first clinical trial to demonstrate statistical significance and a clinically meaningful effect in patients with NPC. These results provide further momentum for the broad clinical development program planned for IB1001 which will address high unmet medical needs for the treatment of both rare and common neurological disorders.

Impact of the GM2 Gangliosidosis Clinical Study

"The results of this study are hugely important for the GM2 community," said Dr. Susanne Schneider, Principal Investigator and Professor of Neurology from the Ludwig Maximilian University of Munich. "IB1001 is the first drug to demonstrate a statistically significant and clinically meaningful effect for the treatment of GM2 Gangliosidosis. IB1001 has a very compelling safety profile, easy oral administration [sachet mixed with water], affirming its very favourable risk/benefit profile as a treatment for this devastating disease."

In a joint statement, Rick Karl, President of the Cure Tay-Sachs Foundation and Dan Lewi, Chief Executive Officer of the Cure Action for Tay-Sachs Foundation, commented: "This treatment is a major breakthrough for the GM2 Gangliosidosis community that includes Tay-Sachs and Sandhoff. It is the first drug to offer hope to the patients and families affected by these devastating diseases. They are progressive, life-threatening conditions with no approved medicinal treatments. There is an urgent need for this effective treatment to be approved and made available for patients in our community before the window of therapeutic opportunity is lost."

“IntraBio understands the importance and urgency of making IB1001 available to GM2 patients as quickly as possible”, said Jim Meyers, President & CEO of IntraBio. “We will continue to engage with regulatory authorities with respect to expediting the pathway to approval”.

IntraBio IB1001 Development

In addition to Clinical Study IB1001-202, IntraBio has completed a parallel multinational clinical trial with IB1001 for the treatment of Niemann-Pick disease Type C (NPC; NCT03759639). In September 2020, IntraBio announced the positive results of this trial (IB1001-201), which met both its primary and secondary endpoints and demonstrated a statistically significant and meaningful improvement in patients with NPC. The results of the trial have been peer-reviewed and published in the Journal of Neurology.

IntraBio is currently conducting a parallel clinical trial for IB1001 for Ataxia-Telangiectasia (A-T; NCT03759678).

Mallory Factor, IntraBio 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 GM2 Gangliosidosis, Niemann-Pick disease Type C, and Ataxia-Telangiectasia, we will continue to investigate IB1001 for other neurological disorders with high unmet medical needs."

About GM2 Gangliosidosis

GM2 Gangliosidosis affects an estimated 1:200,000 -320,000 live births and are caused by mutations in the HEXA gene, which disrupts the activity of the enzyme beta-hexosaminidase A, preventing the enzyme from breaking down GM2 gangliosides. As a result, GM2 gangliosides accumulate to toxic levels, particularly in neurons in the brain and spinal cord, leading to cell death and resulting in the signs and symptoms of Tay-Sachs and Sandhoff disease. There is nothing medically available for the treatment of GM2 Gangliosidosis at this time.

About IB1001-202 Trial

IB1001-202 (NCT03759665) is a multinational clinical trial evaluating IB1001 for the treatment of adult and pediatric patients with GM2 Gangliosidosis. Patients aged 6 years and older were enrolled at trial sites in the United States, the United Kingdom, the European Union.

IB1001 was assessed during 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 for examining symptomatic relief. In the "Extension Phase", patients receive treatment with IB1001 for 1 year. 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 has 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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