



***IntraBio Reports Statistically Significant and Clinically Meaningful Improvements in the Use of IB1001 For Treatment of GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease)***

*~ Multinational clinical trial is the first successful clinical trial for GM2 Gangliosidosis; favourable safety and efficacy data consistent with previously announced IB1001 clinical trial results for NPC ~*

**OXFORD, UK. August 19, 2021:** IntraBio Inc today announced positive data from its multinational clinical trial of IB1001 for the treatment of GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease).

IB1001 demonstrated a statistically significant and clinically meaningful improvement in symptoms, functioning, and quality of life in both the primary and secondary endpoints for pediatric and adult patients with GM2 Gangliosidosis.

The trial met its primary endpoint, the Clinical Impression of Change in Severity (CI-CS), which was assessed by blinded, centralized raters (professors of neurology with expertise in movement and neurological disorders).

The trial also met secondary endpoints including the Scale for the Assessment and Rating of Ataxia (SARA), the Modified Disability Rating Scale (mDRS), the Investigators', Caregivers', and Patients' Clinical Global Impression of Change (CGI-C) assessment.

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

“The results of this study are hugely important for the GM2 community,” said Dr. Susanne Schneider, Principal Investigator and Professor of Neurology from 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.”

Professor Antony Galione, FRS, FMedSci, Statutory Professor of Pharmacology, University of Oxford commented, “GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease) is a devastating disease that has never had any available treatment. We are very excited that IB1001 is the first drug that is effective for this disorder and will improve the lives of so many patients and their families. Given what is known about IB1001’s mechanism, and its multiple successful clinical trials, we will continue to investigate this drug for other rare genetic neurological diseases and for more common neurodegenerative diseases prevalent in society with large unmet medical needs.”

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.”

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.

### **About IB1001-202 Trial**

IB1001-202 (NCT03759665) is a multinational clinical trial evaluating IB1001 for both symptomatic and neuroprotective, disease-modifying treatment for 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.

To investigate its symptomatic effects, 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 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.

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.

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

### **About IB1001**

IB1001, N-acetyl-L-leucine, is an orally administered modified amino acid. *In vivo* studies have identified N-acetyl-L-leucine to be the active isomer of N-acetyl-DL-leucine that can restore neuronal function and protect against/delay disease progression in multiple neurological circuits of the brain. The mechanism of N-acetyl-L-leucine is multi-modal, including altered glucose and antioxidant metabolism, reduced lysosomal storage, and the reduction of neuroinflammation in the cerebellum, leading to the attenuation of cell death.



IntraBio has received Orphan Drug Designations for Acetyl-Leucine from the US Food and Drug Administration (FDA) and the European Commission for the treatment of NPC, GM2, A-T, and Spinocerebellar Ataxias (40+ subtypes). In addition, Acetyl-Leucine has been granted Rare Pediatric Disease Designations for NPC, GM2, and A-T, and Fast Track Designations for NPC and GM2 by the US FDA.

### **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 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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