Therapeutic Update

IntraBio is continuing the development of its lead drug candidate N-acetyl-L-leucine (IB1001) for the treatment of Niemann-Pick disease type C (NPC). IB1001 is an orally administered (sachet) that has been demonstrated to be safe, well-tolerated, and effective for the treatment of NPC.

Positive Phase IIb Multinational Clinical Trials

IB1001-201 (NPC)

In September 2020, IntraBio announced the successful results from multinational Phase IIb trial with N-acetyl-L-leucine (IB1001) for NPC (Clinical Trial IB1001-201, NCT03759639), which demonstrated a statistically significant and clinically meaningful effect on both its primary and secondary endpoints.

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

IB1001 was safe and well-tolerated, with no serious adverse reactions.

The Principal Investigator's publication detailing the complete trial results has been peer-reviewed and published in the Journal of Neurology [Bremova et al. 2021].

IB1001-202 (GM2)

In August of 2021, IntraBio announced the positive results from a second, parallel clinical trial with IB1001 for the related lysosomal storage disorder GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease) (Clinical Trial IB1001-202; NCT03759665). As in the NPC trial, IB1001 met its primary and secondary endpoints and was very safe and well-tolerated. These results, consistent with the IB1001-201 trial for NPC, provide further robust evidence of IB1001's safety and efficacy for rare lysosomal storage disorders.

Supportive Pre-Clinical and Clinical Evidence

The findings from these clinical trials are consistent with a robust body of pre-clinical and clinical evidence demonstrating the symptomatic, and disease-modifying, neuroprotective effects of the drug [Kaya et al. 2021; Bremova et al. 2020; Kaya et al. 2020; Cortina-Borja et al. 2018; te Vrutche et al. 2019; Bremova et al. 2015].

In addition, IntraBio has previously been granted Rare Pediatric Disease Designation and Fast-Track designation for IB1001 for NPC and GM2 by the US FDA, as well as 10 Orphan Drug Designations by the US FDA and EU EMA, including for NPC and GM2.

Pivotal Clinical Trial

Based on the robust evidence demonstrating IB1001's positive benefit/risk profile for NPC, IntraBio is initiating a Phase III pivotal trial with IB1001 for NPC (Clinical Trial IB1001-301).

The trial is a randomized, double-blinded, placebo-controlled, crossover study that will enroll patients 4 years and older at approximately 14 trial sites in 8 different countries, including in Australia, Europe, the United Kingdom, and United States.

During the trial, all patients will receive treatment with IB1001 and placebo in two different “treatment periods” each lasting approximately 12-weeks each. The total duration of the study (with the screening period) will be approximately 26 weeks, during which there will be 6 study visits to the trial site.

Patients who complete the study will be eligible to join an open-label extension phase, where they will receive treatment with IB1001.

Patients will be reimbursed for reasonable out-of-pocket expenses such as travel expenses that will be incurred for participating in the trial.

For further information on the IB1001-301 clinical trial or questions contact:

Cass Fields
Vice-President External Affairs
ccfields@intrabio.com
www.intrabio.com