IntraBio’s lead drug series, IB1000s, are a set of orally administered, modified amino acids (N-Acetyl-Leucine) characterized by a well-established safety and tolerability profile, and has demonstrated statistically significant efficacy in compassionate-use trials for a broad spectrum of lysosomal storage disorders (LSD) and rare and common neurodegenerative diseases.

Based on the pre-clinical studies with the compounds N-Acetyl-DL-Leucine, N-Acetyl-D-Leucine, and N-Acetyl-L-Leucine, IntraBio is prioritizing the development of N-Acetyl-L-Leucine (IB1001), which is believed to have superior clinical benefits over the racemic mixture and D-isomer.

Given the extremely high, unmet medical need, IB1001 is initially being developed for three orphan indications where there are currently no FDA approved therapies: Niemann-Pick Disease Type C (NPC), GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease), and inherited Cerebellar Ataxias (CA).

**Clinical Development**

In compassionate use studies, IB1000s have demonstrated statistically significant improvement in key, clinically-validated neurological scales in patients with NPC, Tay-Sachs disease, and inherited Cerebellar Ataxias (as well as additional LSDs and neurodegenerative diseases). These findings have been significantly supported in additional in vitro and in vivo studies, and are described in multiple published peer-review papers.

**Regulatory History**

IntraBio has been granted eight Orphan Drug Designations (US Food and Drug Administration)/ Orphan Medicinal Drug Designations (European Commission) for IB1000s for the treatment of NPC, GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease), Spinocerebellar Ataxias (of which there are over 40 known subtypes) as well as Ataxia-Telangiectasia.

IntraBio has also been granted two Rare Pediatric Disease Designation for IB1000s by the FDA for the treatment of NPC and GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease). These Rare Pediatric Disease Designation makes IB1000s eligible for, and expedites the request of, a Rare Pediatric Disease Priority Review Voucher (PRV) granted at the time of marketing approval.