Dear Members of the GM2 Gangliosidoses Community,

The U.S. Food and Drug Administration (FDA) has accepted IntraBio's supplemental New Drug Application (sNDA) for review for AQNEURSA™ (levacetylleucine, IB1001) for the treatment of GM2 Gangliosidoses.

The application has been granted Priority Review and has an action date of April 4, 2025, which the FDA can change.

This is the first marketing application for GM2 Gangliosidoses ever accepted in the U.S. The filing of the sNDA for GM2 Gangliosidoses follows FDA approval of AQNEURSA™ (levacetylleucine) on September 24, 2024, for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg.

Kathy Flynn, Chief Executive Officer of the National Tay-Sachs and Allied Diseases Association, shared: "An FDA-approval of AQNEURSA could be life-changing to patients and families living with Tay-Sachs or Sandhoff disease. While we know this application is a first step in the process, we are incredibly motivated and committed to continuing to advocate for safe and effective treatments to benefit patients and families in the GM2 community. NTSAD is proud to have partnered with IntraBio and supported their efforts throughout the development process, and as a community, we stand fully behind their application."

"IntraBio understands the urgency and extremely high medical need for safe and effective treatments for GM2 Gangliosidoses," said IntraBio's Chief Executive Officer, Mallory Factor. "We recognize the significant challenges faced by patients and their families living with these rare and devastating diseases, and we remain deeply committed to bringing this therapy to those who need it most."

We thank the community for the support of this application.

Cass Fields
Vice-President of External Affairs
IntraBio Inc.