IntraBio Announces U.S. FDA Accepts New Drug Application for IB1001 for the Treatment of Niemann-Pick disease Type C

- IntraBio's IB1001 New Drug Application was accepted and granted priority review
- PDUFA date set for September 24th, 2024
- Application based on positive results of the IB1001-301 Phase 3 Pivotal Trial which was recently published in the New England Journal of Medicine on February 1st, 2024

March 26, 2024, 8:00 AM EDT
IntraBio Inc today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for IB1001 for the treatment of Niemann-Pick disease Type C (NPC).

The application has been granted Priority Review and was given a Prescription Drug User Fee Act (PDUFA) target action date of September 24th, 2024.

The NDA is based on the results of a Pivotal Phase 3 trial (IB1001-301) for adult and pediatric patients with NPC that met all primary and key secondary endpoints and showed IB1001 improved neurological signs and symptoms, functioning, and quality of life versus placebo. The findings from the study were recently published in the New England Journal of Medicine on February 1st, 2024.

The NDA also included data from a positive multinational Phase IIb trial of IB1001 for NPC, which also met its primary and secondary endpoints and showed improvement in symptoms, functioning, and quality of life in pediatric and adult patients with NPC with the drug being well-tolerated.

Sean Kassen, Director of the Ara Parseghian Medical Research Fund commented: "We are absolutely thrilled and filled with hope with the news of IntraBio's NDA filing acceptance. NPC has an extremely high unmet medical need and the data from the IB1001 clinical trials demonstrated that this therapy would bring relief and improved quality of life to those affected by Niemann-Pick Type C disease. We are excited and committed to working with and supporting the NPC community and IntraBio and look forward to the eventual approval of IB1001 so all patients will benefit from this therapy."

"The FDA's acceptance of IntraBio's NDA submission for IB1001 and granting of priority review brings us one step closer to our ultimate goal of delivering new, effective treatments to patient communities like NPC who have extremely high unmet medical needs," said Mallory Factor, IntraBio's Executive Chairman. "IntraBio remains dedicated to advancing therapies and addressing unmet medical needs, and we are optimistic IB1001 will be approved and made rapidly available for all NPC patients."

Professor Elizabeth Berry-Kravis, Rush University Medical Center, Chicago, IL, commented: "We are tremendously excited to have potential new treatment options for our NPC patient community. Once approved, we intend to treat all our NPC patients who meet the labelling criteria with IB1001 and to carefully track long-term improvement relative to baseline with whatever therapies they are on to help elucidate the long-term effects."

IntraBio also today announced it has recently closed an equity financing round, in which it raised over $40 million US, to support the commercialization and launch of IB1001 subject to FDA approval.

About IB1001

IB1001 is an orally administered therapy that is taken up by monocarboxylate transporters, which are expressed ubiquitously and deliver IB1001 to all tissues, including across the blood–brain barrier. Inside cells, IB1001's underlying mechanism of action is multi-modal and targets the major drivers of disease pathophysiology in rare and common neurological disorders.

About IntraBio

IntraBio Inc, a US biopharmaceutical company, is focused on the development of novel drugs addressing rare and common neurological diseases. IntraBio's platform technologies result from decades of research and collaboration with universities and institutions worldwide. Its clinical programs are based upon the expertise in lysosomal function and intracellular signalling of its scientific founders from the University of Oxford and the University of Munich.