

IntraBio Inc. is pleased to confirm that the Clinical Trial IB1001-201, “*Effects of N-Acetyl-L-Leucine on Niemann-Pick type C Disease (NPC): A multinational, multi-center, open-label, rater-blinded Phase II study*” is open for US recruitment at the Mayo Clinic.

“IntraBio’s IB1001-201 trial is open for recruitment in the United States,” said Mallory Factor, Chairman, IntraBio, “US patients will now have the opportunity to access the novel therapy IB1001 which we believe will significantly improve their quality of life”.

IB1001-201 is a multinational study that investigates the effects of the novel drug IB1001 (N-Acetyl-L-Leucine) for treatment of Niemann-Pick Disease type C (NPC). The trial has been accepted by the US Food and Drug Administration (IND 134369) as well as National Regulatory Agencies in the UK, Germany, Spain, and Slovakia (EudraCT: 2018-004331-71).

In the United States, patients aged 18 and older with a genetically confirmed diagnosis of NPC may be eligible for recruitment. It is planned that patients enrolled in the trial will be assessed during three study phases: a baseline period, a 6-week treatment period, and a 6-week post-treatment washout period. Patients (and a caregiver) will be reimbursed for reasonable out-of-pocket expenses incurred for participating in the trial, such as travel and parking.

“We are very excited to be actively enrolling patients,” said Dr Anhar Hassan, Principal Investigator at Mayo Clinic. “We look forward to recruiting patients immediately and investigating this novel treatment which we hope can help address the NPC community’s extremely-high medical need.”

Patients and families who are interested in learning more about the trial, including the eligibility criteria and enrollment process, should contact the Mayo Clinic Study Team:

**The Mayo Clinic**  
**Rochester, MN 55905**  
**Principal Investigator: Anhar Hassan, MD**  
**Contact: Sandra Looney, Clinical Research Coordinator**  
**Email: [Looney.Sandra@Mayo.edu](mailto:Looney.Sandra@Mayo.edu)**  
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For the complete enrollment criteria as well as details regarding the study assessments, multinational clinical trial sites, etc., please visit [ClinicalTrials.Gov \(NCT03759639\)](https://ClinicalTrials.gov/NCT03759639).

#### **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 clinical programs leverage the expertise in lysosomal function and intracellular calcium signaling of its scientific founders and advisors from the University of Oxford, the University of Munich and other premier universities and institutions worldwide.

In addition to Clinical Study IB1001-201, IntraBio is conducting two additional multinational clinical trials involving IB1001 for the treatment of GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease) and Ataxia-Telangiectasia (A-T).

IntraBio Inc. is a US corporation with its principal laboratories and offices in Oxford, United Kingdom. For further information please contact:

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