



IB1001-201 (NPC): NOV. 2019 RECRUITMENT UPDATE

IntraBio Ltd. is pleased to share that the IB1001-201 clinical trial has screened two-thirds ($\frac{2}{3}$) the target number of patients.

The study, which investigates N-acetyl-L-leucine (IB1001) for the treatment of Niemann-Pick disease Type C (NPC), will enroll a total of approximately 30 patients across all international sites. Since recruitment commenced in September 2019, twenty (20) patients have been screened across nine (9) international clinical trial sites.

Recruitment is expected to continue rapidly and be completed by December 2019.

To ensure all patients have the opportunity to participate, interested patients are encouraged to contact their nearest study center as soon as possible to schedule a screening visit before enrollment is complete.

Information on each site, including contact details, can be found below.

GERMANY

Ludwig Maximilian University of Munich

Patients aged 6 Years +

Principal Investigator: Susanne Schneider, MD

Contact: Anita Hennig, MD

Email: Anita.Hennig@med.uni-muenchen.de

Tel: +49(0)89 4400 76777

University of Giessen

Patients aged 6 Years +

Principal Investigator: Andreas Hahn, MD

Contact: Kyriakos Martakis, MD

Email: Kyriakos.Martakis@paediat.med.uni-giessen.de

Tel: +49 (0)641 985 43543

SLOVAKIA

Comenius University in Bratislava

Patients aged 6-17 Years

Principal Investigator: Miriam Koníková, MUDr, PhD

Sub-Investigator: Tomáš Foltán, MUDr

Contact: Katarína Cichovská

Email: katarina.cichovska@dfnsp.sk

Tel: +421 2 59371 274

SPAIN

Bellvitge University Hospital

Patients aged 6 Years +

Principal Investigator: Jordi Gascon Bayarri, MD

Email: jordigneuro@bellvitgehospital.cat

Tel: +34 932 607500 ext 2958

UNITED KINGDOM

Great Ormond Street Hospital

Patients aged 6 – 17 Years

Principal Investigator: Paul Gissen, MD

Contact: Olivia Rosie-Wilkinson, Senior Research Coordinator

Email: Olivia.Rosie-Wilkinson@gosh.nhs.uk

Tel: +44 (0)2074 059200 ext. 47267

Royal Manchester Children's Hospital

Trial Site for Patients Aged 6 – 17 Years

Principal Investigator: Simon Jones, MD

Contact: Laura Crowther, Clinical Trials Manager

Email: laura.crowther@mft.nhs.uk

Tel: +44 (0)161 701 9137

Royal Free London NHS Foundation Trust

Trial Site for Patients Aged 13 Years +

Principal Investigator: Uma Ramaswami, MD

Contact: Masoud Kazemi, Senior Research Lead

Email: masoud.kazemi@nhs.net

Tel: +44 (0) 207 830 2814

Salford Royal NHS Foundation Trust

Trial Site for Patients Aged 18 Years +

Principal Investigator: Reena Sharma, MD

Contact: Marie Meehan, Senior Clinical Research Practitioner/ Team Manager

Email: Marie.Meehan@srft.nhs.uk

Tel: +44 (0)161 206 4192

UNITED STATES

The Mayo Clinic (Rochester, MN)

Patients 18 Years +

Principal Investigator: Anhar Hassan, MD

Contact: Sandra Looney, Clinical Research Coordinator

Email: Looney.Sandra@Mayo.edu

Tel: +1 (507) 538-4107

About IB1001-201

IB1001-201 investigates N-acetyl-L-leucine (IB1001) for the treatment of Niemann-Pick disease Type C (NPC). This international trial is being run in clinical centers in the United States, the United Kingdom, Germany, Spain, and Slovakia. In Europe, patients aged 6 and older with a confirmed diagnosis of NPC may be eligible for recruitment.

The trial will consist of three study phases: a baseline period, a 6-week treatment period, and a 6-week post-treatment washout period followed by a planned one-year extension phase. Patients (and a caregiver) will be reimbursed for reasonable out-of-pocket expenses incurred for participating in the trial, such as travel and parking.

Additional information on Clinical Trial Study IB1001-201 can be found via [ClinicalTrials.Gov\(NCT03759639\)](https://clinicaltrials.gov/ct2/show/study/NCT03759639).