NPC COMMUNITY LETTER OF SUPPORT:
FDA Review of IB1001

APRIL 2024

We are very excited about the important news of IntraBio's New Drug Application (NDA) acceptance by the US Food and Drug Administration (FDA) for N-acetyl-L-leucine (IB1001). Our community is now potentially six months away from having our first drug approved for the treatment of Niemann-Pick type C (NPC) and we remain committed to supporting the application throughout the review process.

The NPC community has been involved in the development of IB1001 from its inception and has collaborated with IntraBio throughout the program to help bring this treatment to patients as quickly as possible.

In multiple clinical trials, IB1001 has demonstrated a statistically significant and clinically meaningful benefit for the debilitating neurological signs and symptoms that severely impact the lives of our patients and families. We are excited and confident in the robustness of the application as it is supported by the strong efficacy and safety results from a double-blinded, randomized, placebo-controlled, crossover design Phase III trial recently published in the prestigious New England Journal of Medicine (NEJM). Dr Cynthia Tifft, in her Expert Perspective Editorial 'N-acetyl-L-leucine and Neurodegenerative Diseases' in the same NEJM issue, described the science behind IB1001 and recognized its potential for the treatment of all neurodegenerative lysosomal storage disorders.

With the extremely high unmet medical need for treatments for NPC, we believe IB1001 can bring those affected and their families much-needed relief and potentially change the course of this devastating disease.

We continue to support the IB1001 program and we remain committed to working with the FDA to ensure the NPC community of patients and families who are in dire need have access to therapies as soon as possible. Patients and families are counting the days until treatments are approved and they can access approved drugs. We look towards the future with renewed optimism and excitement, with two investigational drugs now under FDA review, in hopes of approval and access to all who could benefit.

On behalf of the those living with NPC and their direct caregivers in the United States:

Ara Parseghian Medical Research Fund, University of Notre Dame

DART — Dana's Angels Research Trust ...aiming to cure Niemann-Pick Type C

Firefly Fund — Shining a Light on NPC1

hadleyhope

Hide & Seek Foundation for lysosomal disease

Hope for Marian

nnpdf — National Niemann-Pick Disease Foundation, Inc.

SOAR — Support Of Accelerated Research for Niemann-Pick Type C