

To the CACNA1A Community,

On behalf of IntraBio, I am so pleased to formally introduce our company and to share an update on our clinical development program for CACNA1A disorders.

IntraBio Inc. is a US-based biopharmaceutical company focused on the discovery, development, and commercialization of innovative, novel therapies for neurodegenerative and neurodevelopmental disorders with high unmet medical needs. Our lead investigational therapy, IB1001 (levacetylleucine), is an orally-administered small molecule that has been investigated for the past decade, and has demonstrated clinical benefit across various rare and common neurological conditions, including multiple neurological disorders in which cerebellar ataxia is a primary and debilitating symptom.

IB1001 is approved by the US Food and Drug Administration (FDA) [commercially known as AQNEURSA (levacetylleucine)] for the treatment of neurological manifestations of Niemann-Pick disease Type C (NPC). NPC is an inherited lysosomal storage disorder which features cerebellar ataxia, gait and movement dysfunction, dystonia, ocular motor disturbances, and seizures. In the pivotal clinical trial for NPC, IB1001 significantly improved these neurological symptoms, as well as functioning and quality of life, in both pediatric and adult patients. IB1001 was well-tolerated, with no serious adverse reactions.

Currently, IntraBio has separate development programs with IB1001 ongoing for other rare diseases, including GM2 Gangliosidosis and Ataxia-Telangiectasia (A-T).

We are excited to share that IntraBio is preparing to initiate a Phase III clinical trial (“IB1001-304”) with IB1001 for CACNA1A-related disorders in the second half of 2026. The Investigational New Drug (IND) application for the study is active with the FDA, and clinical trial sites in the United States and Europe are currently being identified. Information on the design and inclusion criteria of this upcoming CACNA1A trial is posted on [ClinicalTrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT07221292) (NCT07221292).

The IB1001-304 clinical trial will be the first-ever clinical trial conducted specifically for CACNA1A-related disorders, and we are so fortunate to partner with organizations like the CACNA1A Foundation to share information about the study, and support families throughout the program. As with all of our programs, we are committed to working in close alignment with patients, families, clinicians, and researchers throughout the CACNA1A community. We understand that families and clinicians will have many questions as this program progresses. IntraBio is committed to keeping the CACNA1A Foundation and its community informed and updated as we move toward the initiation of the clinical study.

On behalf of all of us at IntraBio, we thank you in advance for your support as we work towards a shared goal of bringing novel treatments to individuals with CACNA1A disorders, and bettering the lives of CACNA1A patients and families, as quickly as possible.

Best,
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