

Global Update – CLN5 Batten Disease Clinical Trial

August 12, 2024 – On Friday, August 9, Neurogene Inc. announced enrollment is now complete for its Phase 1/2 investigational trial of NGN-101 Gene Therapy for the treatment of CLN5 Batten disease, with a total of six patients enrolled (Clinical trial ID: [NCT05228145](#)). Neurogene unveiled this news in its *Second Quarter 2024 Financial Results and Highlights Recent Updates*.

According to the report, Neurogene plans to provide interim clinical data and a regulatory update in the first quarter of 2025.

“Given the rarity of CLN5 Batten disease, FDA alignment on a streamlined registrational pathway will be critical for continued investment in the program,” the report noted.

“There is no denying we’ve experienced tumultuous times for rare disease drug development in recent years, and so positive news like this not taken for granted,” said Ineka Whiteman PhD, Head of Research & Medical Affairs for BDSRA Foundation and BDSRA Australia. “We are excited to see the CLN5 disease gene therapy program continue hitting new milestones, and we have assured Neurogene of our ongoing support and collaboration to ensure this program continues toward the best possible outcome.”

Global patient advocacy organizations for the Batten disease community look forward to providing further updates on this study and continuing their global collaboration to keep the Batten community informed and to advocate for treatments and cures for all Batten disease CLN types.

Read Neurogene’s full report by clicking [here](#).



What is Batten disease?

- Batten disease, or Neuronal Ceroid Lipofuscinoses (NCL), is a family of rare, genetic neurodegenerative conditions primarily affecting children. Genetic mutations disrupt the cells' ability to dispose of waste, leading to a build-up of protein and lipid deposits eventually leading to cell death. Collectively, NCLs are among the most prevalent inherited pediatric neurodegenerative disorders worldwide, with an incidence of between 1 and 7 per 100,000 live births.

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