



## Neurogene Announces EMA Grants Orphan Drug Designation to CLN5 Batten Disease Gene Therapy

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*EMA's Orphan Drug Designation for CLN5 Batten Disease Therapy Follows FDA's Orphan Drug Designation*

*Designation Demonstrates Continued Progress in Establishing Batten Disease Franchise*

**NEW YORK, June 29, 2021** – [Neurogene Inc.](#), a company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, today announced that the European Medicines Agency (EMA) has granted Orphan Drug Designation (ODD) to its adeno-associated virus (AAV) encoding a codon-optimized human CLN5 transgene (hCLN5) for patients diagnosed with CLN5, a subtype of Batten disease.

"Signs and symptoms of CLN5 disease include vision loss, cognitive and motor impairment, and seizures. The disease often takes a rapidly progressive course within a few years of symptom onset, leading to premature death. With no available treatment options, the outcome is devastating for children and their families," said Angela Schulz, M.D., Ph.D., Children's Hospital, NCL Specialty Clinic, International Center of Lysosomal Storage Disorders, University Medical Center Hamburg-Eppendorf.

"This regulatory designation is an important milestone toward addressing the urgent, unmet need of families and patients living with CLN5, and follows the Orphan Drug Designation previously granted in 2020 by the U.S. Food and Drug Administration (FDA) for this same gene therapy candidate," said Rachel McMinn, Ph.D., Founder and CEO of Neurogene. "Neurogene is collaborating with Batten disease experts, regulatory authorities, and caregivers to provide a safe and effective gene therapy for this disease as quickly as possible."

### About Orphan Drug Designation

EMA orphan designation is designed to encourage the development of new treatments for life-threatening or chronically debilitating conditions that are rare (affecting not more than five in 10,000 people in the European Union) and have no approved treatment options currently available. Medicines that meet the EMA's orphan designation criteria qualify for several incentives, including 10 years of market exclusivity, protocol assistance, and potentially reduced fees for regulatory activities. Applications for orphan designation are examined by the EMA's Committee for Orphan Medicinal Products (COMP), using the Committee's network of experts.

### About CLN5, a subtype of Batten disease

Batten disease lies within the family of lysosomal storage disorders (LSDs). LSDs are progressive, multisystemic, inherited metabolic disorders associated with premature death. Batten disease, also called neuronal ceroid lipofuscinoses (NCL), is a family of rare neurodegenerative diseases caused by pathogenic changes in one of a series of genes that results in the accumulation of toxic deposits across multiple organ systems, including the brain, eye, skin and others. CLN5 is a rare, pediatric-onset and rapidly progressive late infantile subtype caused by a variant in the CLN5 gene. It is characterized by loss of vision, seizures, and progressive decline in intellectual and motor capabilities beginning in childhood. Diagnosis of the disease is confirmed through genetic testing. Neurogene has developed an educational website, [www.LivingBatten.com](http://www.LivingBatten.com), to support patients, families and care providers looking for more information, resources, and genetic testing.

### About Genetic Testing

Neurogene is committed to lowering the barriers of obtaining a genetic diagnosis for patients and has partnered with Invitae to co-sponsor two genetic testing programs in the US and in Canada. Healthcare providers can order, at no cost, the Detect Lysosomal Storage Diseases panel for patients who are suspected of having a lysosomal storage disease. In addition, the Invitae Epilepsy panel is available at no charge for any child under the age of eight who has had an unprovoked seizure. The Detect Lysosomal Storage Diseases panel is available at no charge to patients who are suspected of having a lysosomal storage disease. CLN5 and other Batten disease genetic variants are available on both genetic testing panels. Visit <https://www.invitae.com/en/sponsored-testing/> for more details.

### About Neurogene Inc.

Neurogene Inc. is focused on developing life-changing genetic medicines for patients and their families affected by rare, devastating neurological diseases. We partner with leading academic researchers, patient advocacy organizations and caregivers to bring therapies to patients that address the underlying genetic cause of a broad spectrum of neurological diseases where no effective treatment options currently exist. Our lead programs use adeno-associated virus (AAV) vector-based gene therapy technology to deliver a normal gene to patients with a dysfunctional gene. Neurogene is also developing novel gene therapy technologies to advance treatments for complex neurological diseases that conventional gene therapy cannot successfully address. For more information, visit [neurogenestg.wpengine.com](http://neurogenestg.wpengine.com).

### Media Contact

Linda Phelan Dyson, MPH  
[linda.dyson@neurogene.com](mailto:linda.dyson@neurogene.com)  
973-986-5973