Neurogene Announces FDA Clearance of IND for NGN-101 Gene Therapy to Treat CLN5 Batten Disease

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Planned Phase 1/2 Gene Therapy Trial Will Be First to Investigate Treatment of Both Neurodegenerative and Ocular Disease Manifestations of Batten Disease

Trial Expected to Initiate in First Half of 2022

NEW YORK, September 14, 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 U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application for NGN-101. NGN-101, Neurogene's lead product candidate in its Batten disease franchise, is an investigational adeno-associated virus (AAV) gene therapy for the treatment of CLN5 Batten disease, a rare and fatal neurodegenerative disorder with no approved disease-modifying therapies available.

"NGN-101 is the first investigational gene therapy developed to address the devastating consequences of both neurodegeneration and vision loss in patients living with Batten disease. Importantly, we believe our preclinical data strongly support the potential of NGN-101 to stop the progression of CLN5 Batten disease, including the associated vision, motor, cognitive, and behavioral declines," said Rachel McMinn, Ph.D., Founder and CEO of Neurogene. "FDA clearance of our first IND is an incredibly significant milestone for Neurogene, and moves us one step closer to delivering on our mission to bring transformative treatments to patients living with rare neurologic diseases."

IND clearance enables Neurogene to initiate a Phase 1/2 trial to assess the safety, tolerability, and efficacy of NGN-101 in patients with CLN5 Batten disease. The planned open-label, single-arm trial will evaluate a single dose delivered via intracerebroventricular (ICV) and intravitreal (IVT) routes of administration. University of Rochester will serve as the lead trial site in the United States, led by Jonathan W. Mink, M.D., Ph.D., the Frederick A. Horner MD Distinguished Professor in Pediatric Neurology, Chief of Child Neurology, and Director of the University of Rochester Batten Center.

"CLN5 is a devastating and rapidly progressive neurodegenerative disease in children that leads to vision loss, cognitive and motor impairment, seizures and, ultimately, premature death," said Jonathan W. Mink, M.D., Ph.D. "This trial of NGN-101 will move research forward in developing a potentially disease-modifying treatment for CLN5 disease, providing hope to individuals and families where currently none exists."

About NGN-101
NGN-101 is being developed as a one-time treatment via intracerebroventricular (ICV) and intravitreal (IVT) routes of administration for CLN5 Batten disease using an adeno-associated virus (AAV) to deliver the gene encoding CLN5, which is deficient in children with CLN5 Batten disease. Preclinical data strongly suggest that NGN-101 treatment has the potential to halt the key features of disease progression in patients with CLN5 Batten disease, including the associated vision, motor, cognitive, and behavioral declines. NGN-101 has received Orphan Drug Designation by the U.S. and European regulatory agencies.

About CLN5, a subtype of Batten disease
Batten disease is a group of rare, inherited neurodegenerative diseases also called neuronal ceroid lipofuscinoses (NCLs), in which a pathogenic genetic change in one of 13 different genes results in the accumulation of toxic deposits across multiple organ systems, culminating in progressive deterioration in intellectual and motor capabilities, loss of vision, and premature death. The different subtypes of Batten disease share many common features, and a diagnosis requires genetic testing to confirm the cause of disease. CLN5 Batten disease is caused by a pathogenic variant in the CLN5 gene, and is characterized by loss of vision, seizures, and progressive decline in intellectual and motor capabilities with initial signs and symptoms starting in childhood. Neurogene has developed an educational website, www.LivingBatten.com, to support patients, families, and care providers looking for more information, resources, and genetic testing.

About Neurogene Inc.
Neurogene's mission is to develop life-altering genetic medicines for patients and their families affected by rare, devastating neurological diseases. Our lead programs use adeno-associated virus (AAV) vector-based gene therapy technology to deliver a normal gene to patients with a dysfunctional gene. 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. 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.

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