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FDA Grants Orphan Drug Designation to Neurogene's Gene Therapy for the Treatment of CLN5 Batten Disease

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Orphan Drug Designation granted to Neurogene's adeno-associated virus vector with engineered transgene encoding the human CLN5 gene

NEW YORK, July 7, 2020 – Neurogene Inc., a company founded with a mission 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) granted Orphan Drug Designation to adeno-associated virus vector with engineered transgene encoding the human CLN5 gene for patients with CLN5, a form of Batten disease. Batten disease, a common name for a rare class of diseases called neuronal ceroid lipofuscinoses (NCL), affects an estimated 2-4 out of every 100,000 children in the United States.

"CLN5 is a devastating neurodegenerative disease with no FDA approved treatment options," said Rachel McMinn, Ph.D., Neurogene's Founder and Chief Executive Officer. "Receiving Orphan Drug Designation from the FDA is an important regulatory milestone, and we look forward to advancing our gene therapy program into the clinic."

The FDA grants Orphan Drug Designation to drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases or conditions affecting fewer than 200,000 people in the United States. Orphan Drug Designation provides benefits to drug developers designed to support the development of drugs and biologics for small patient populations with unmet medical needs. These benefits include assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of marketing exclusivity.

About CLN5

organ systems, including the brain, eye, skin and other tissues. The most prominent effects occur in the brain, where the progressive and inevitable loss of neurons lead to devastating declines in cognitive and motor function in those with Batten disease. The subtype CLN5 is a rare, pediatric-onset and rapidly progressive disease caused by defects in the CLN5 gene. CLN5 disease is characterized by progressive deterioration in intellectual and motor capabilities and vision loss, as well as seizures and death in childhood or adolescence. Diagnosis of the disease is confirmed through genetic testing. Currently, there are no approved disease-modifying therapies available.

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. Healthcare providers can order, at no charge, an Invitae Epilepsy panel for any child under the age of eight who has had an unprovoked seizure, or the Detect Lysosomal Storage Diseases panel for patients suspected of having a lysosomal storage disease. 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 exist today. Our lead programs are designed to use AAV-based gene therapy technology to deliver a normal gene to patients with a dysfunctional gene. Neurogene is also investing in novel technology to develop treatments for diseases not well served by gene therapy. For more information, visit Neurogene Inc..

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