Neurogene Offers Access to Genetic Testing to Patients Suspected of Having a Lysosomal Storage Disease

July 25, 2019

Neurogene joining with Invitae to reduce barriers to genetic testing for more than 50 lysosomal storage diseases

Initiative is designed to identify the many patients with AGU that are currently misdiagnosed or undiagnosed

NEW YORK, July 25, 2019 – Neurogene Inc., a company founded with a mission to bring life-changing medicines to patients and families affected by rare neurological diseases, today announced it has become the founding sponsor of a new program called "Detect Lysosomal Storage Diseases (LSDs)" from Invitae, a leading medical genetics company. With support from Neurogene for this program, Invitae offers genetic testing and counseling at no charge to patients suspected of having a lysosomal storage disease, or LSD.

"As Neurogene works to find an effective gene therapy for patients and their families living with an LSD called AGU, we are also taking critical steps to learn more about the disease and identify patients," said Rachel McMinn, Ph.D., Neurogene's Founder and Chief Executive Officer. "We are currently enrolling patients in a natural history study of AGU and are planning IND-enabling studies in order to move a treatment into a first-in-human clinical trial as expeditiously as possible. We are excited about this new collaboration with Invitae, as we hope easier access to genetic testing will empower more families currently searching for a diagnosis."

Known LSDs are all progressive, multisystemic, inherited metabolic diseases associated with premature death. Neurogene is developing a treatment for a rare neurodegenerative LSD called aspartylglucosaminuria (AGU), in which all patients experience some form of developmental delay among other symptoms such as progressive intellectual disability, respiratory infections and gait disturbance. AGU is commonly misdiagnosed or undiagnosed in the majority of patients. This new testing program includes a specific LSD testing panel of 53 genes designed to provide patients and families accurate information quickly to preserve valuable treatment time. In addition, the program offers three more general panel options — ranging from 23 to 123 genes — as well as single genes that can be ordered.

"Access to no-charge genetic testing opens doors for patients and families who are constantly searching for answers, have received a misdiagnosis or remain undiagnosed," said Eeva Rantanen, Chairman of the AGU Finnish advocacy association, Suomen AGU ry. "Neurogene's support of this program creates the opportunity for families to connect with others in the AGU community and know they are not alone."

"Having partners like Neurogene join us in this important effort is critical to our goal of helping provide a clear diagnosis for children suspected of having an LSD," said Katherine Stueland, chief commercial officer of Invitae. "As we seek to break down the barriers to genetic testing for diagnosing disease and informing treatment options, this collaboration holds great potential for families and physicians looking for answers."

Additional details, as well as terms and conditions of the program, can be found at https://www.invitae.com/en/detectLSDs/.

About Aspartylglucosaminuria (AGU)

AGU is a rare, neurodegenerative lysosomal storage disorder caused by a deficiency of the aspartylglucosaminidase (AGA) enzyme, which leads to toxic accumulation of N-acetylglucosamines and cellular dysfunction. AGU cases have been reported throughout the world and across ethnicities, and it is most well-studied in people of Finnish descent. AGU is characterized by developmental delay and subsequent intellectual disability, which worsens with age. As disease progresses, patients experience psychomotor regression with worsening gait disturbance, behavioral/emotional issues and worsening intellectual disability. People with AGU have a shortened life span, with mortality typically in the fourth decade. Additional information about AGU can be found at www.LivingAGU.com.

About Neurogene Inc.

Neurogene was founded to bring life-changing medicines to patients and families affected by rare neurological disorders. We partner with leading academic researchers, patient advocacy organizations and caregivers to bring to patients therapies that address the underlying genetic cause of a broad spectrum of neurological diseases where no effective treatment options exist today. Our lead programs 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 neurogenestg.wpengine.com.

About Invitae

Invitae Corporation (NYSE: NVTA) is a leading genetics company whose mission is to bring comprehensive genetic information into mainstream medicine to improve healthcare for billions of people. Invitae's goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time, and lower prices. For more information, visit the company's website at invitae.com.

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