February 10, 2022

Dear Patient Advocacy Organizations,

Neurogene Inc., a biotech company committed to developing life-changing genetic medicines for patients and their families affected by rare neurological disorders, is excited to share an important update on our development program for CLN5, a subtype of Neuronal Ceroid Lipofuscinosis (NCL), or Batten disease. **The clinical trial for the investigational AAV (adeno-associated virus) gene therapy, NGN-101, is now enrolling participants at the clinical trial site, University of Rochester Medical Center in Rochester, NY, USA.**

The information below is available here: NCT05228145

This investigational gene therapy clinical trial is titled: ***A Phase 1/2 Intracerebroventricular and Intravitreal Administration of NGN-101 for Treatment of Neuronal Ceroid Lipofuscinosis (NCL) Subtype 5 (CLN5) Disease.*** This first-in-human gene therapy clinical trial is the first of its kind. Enrolled participants will receive NGN-101 to the brain [through intracerebroventricular (ICV) administration] and the eye [through intravitreal (IVT) administration] to address both the neurodegeneration and vision loss associated with CLN5 Batten disease.

About the Phase 1/2 Gene Therapy Clinical Trial for CLN5 Batten Disease:

- This is a **prospective, open-label** clinical trial
  - This is a clinical trial where all participants will know they are receiving the investigational gene therapy product and will be followed over time.
- This gene therapy clinical trial will enroll children who are **3 to 8 years old** with genetically confirmed Neuronal Ceroid Lipofuscinosis subtype 5 (CLN5) disease (a subtype of Batten disease)
  - In this type of clinical trial, it is important to have the participants similar in age and stage of disease progression to better understand the safety and clinical effects of the investigational gene therapy
- The investigational gene therapy, NGN-101, will be **given as a single intracerebroventricular (ICV) dose into the brain and a single intravitreal (IVT) dose into one eye**
  - Both doses will be given during the same procedure
- **Each participant will be followed for safety and efficacy** 5 years after dosing
  - For approximately the first 6 months after the gene therapy is given, the participant will reside close to the clinical trial site to enable the clinical trial doctor to monitor and care for the participant
  - After the initial safety monitoring period, there will be telephone and in-person visits with the gene therapy clinical trial site in decreasing frequency for regular assessments over a 5-year period
- **There is a comprehensive travel and expense policy** in place covering trial-related costs and expenses
  - Trial-related costs and expenses are paid by Neurogene; more details of the specific policy can be provided by the clinical trial site
- Although the clinical trial site is in the US, **patients from around the world will be considered for enrollment and are encouraged to contact Neurogene for more information at 877-237-5020 (US+1)**
  - Travel and accommodations will be arranged for participants in the clinical trial
  - The clinical trial staff will provide more details
- More information about the clinical trial, including inclusion/exclusion (eligibility) criteria, can be found at clinicaltrials.gov (see link above), from the Patient Advocacy & Engagement team at Neurogene, and from the staff at the clinical trial site

- Participation in this trial is voluntary and does not guarantee results

Educational Resources for Clinical Trials:

- Gene Therapy Clinical Trial Process – American Society of Gene & Cell Therapy (ASGCT) https://patienteducation.asgct.org/gene-therapy-101/clinical-trials-process

Contact Neurogene for more information and to be connected with the clinical trial site:

- Families, physicians, and healthcare providers are encouraged to contact the **Neurogene Contact Center at 877-237-5020 (US+1)** for more detailed information
- **Email: Families** interested in more information about the clinical trial can email Neurogene at: **patientinfo@neurogene.com**
- **Email: Healthcare Providers** interested in more information can email Neurogene at: **medicalinfo@neurogene.com**
- **Neurogene can arrange to have a professional translator available if needed**

Additional information about Neurogene studies:

- Neurogene is also conducting a natural history study that is enrolling patients with CLN5 Batten disease
- Patients who do not participate in the Phase 1/2 Gene Therapy Clinical Trial for CLN5 Batten disease may be eligible for the Natural History Study
- A natural history study is not an interventional clinical trial, and no investigational product is given
- This study is critically important research to better understand the natural course of CLN5 Batten disease
- More information can be found at: https://www.clinicaltrials.gov/ct2/show/NCT03822650?term=neurogene&draw=2&rank=2

Educational Resource for Natural History Studies:

- Understanding Cell & Gene Medicine for your Community https://globalgenes.org/resources/understanding-cell-gene-medicine/

Additional information about Neurogene and the clinical trial can be found here: neurogene.com/patients-and-families

Sincerely,
The Patient Advocacy & Engagement Team at Neurogene

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