May 18, 2022

Dear Rett Syndrome Community,

Today Neurogene announced a new development program for Rett syndrome, NGN-401. NGN-401 is an *MECP2* gene therapy candidate using Neurogene's novel EXACT technology, which was developed in collaboration with the University of Edinburgh. This work at the University of Edinburgh is led by Stuart Cobb, Ph.D., who has been researching Rett syndrome for over 15 years, striving to make a difference for patients and families. Dr. Cobb is the Simons Research Fellow in Neuroscience at the Patrick Wild Centre and Centre for Discovery Brain Sciences at the University of Edinburgh, and also serves as the Chief Scientific Officer at Neurogene. The Rett Syndrome Research Trust (RSRT) introduced Dr. Cobb to Neurogene in 2018. You can read more about Dr. Cobb and RSRT at www.reverserett.org.

NGN-401 is currently in the preclinical stage of development (not in humans). A new preclinical study that Dr. Cobb presented at this year's premier gene therapy scientific forum (ASGCT) showed that NGN-401 demonstrated efficacy while avoiding the toxicity associated with too much *MECP2* expression in a mouse model. Once additional preclinical studies and regulatory requirements are completed, Neurogene will request regulatory approval to progress into clinical trials for females affected by Rett syndrome. Following regulatory clearance, Neurogene will be in a position to advance NGN-401 into a human clinical trial.

We recognize that the Rett syndrome community is eager to know what timelines may be associated with this work. While we do not know the answer at this time, we can assure the community that Neurogene is working with a strong sense of urgency, because we realize that patients and families are waiting for investigational treatments to advance into clinical trials. Neurogene will share available updates on NGN-401 with the patient advocacy organizations, and through our Neurogene Inc. Facebook page.

Neurogene is appreciative of the Rett syndrome patient organizations for their collaboration, including Rett Syndrome Research Trust, International Rett Syndrome Foundation, and Reverse Rett (UK). For more information about each of these organizations, visit https://neurogene.com/patients-and-families/about-rett-syndrome/. As we advance, we look forward to engaging with other Rett syndrome organizations.

Neurogene's press release can be found at Neurogene.com in the "Latest News" section or directly from this link:

https://www.neurogene.com/news/

We look forward to getting to know you, and to learn more about the impact Rett syndrome has on patients and families. We would like to introduce ourselves and provide answers to questions you may have.

Who is Neurogene?

Neurogene is a company founded on the vision to push the boundaries of genetic medicine to address complex and devastating neurological diseases, turning them into treatable conditions and improving the lives of patients and families impacted by rare diseases.

Rachel McMinn, Ph.D., is the founder and Chief Executive Officer of Neurogene. Her life-long dream to help improve the lives of patients and families was inspired by her brother, who lives with an undiagnosed, debilitating rare neurological disease. She dedicated her early career to science, completing a Ph.D. in chemistry and molecular biology, then accepted a post-doctoral fellowship in cell and molecular biology. Rachel spent several years on Wall Street as a biotech equity analyst before she moved into a leadership role in biotech where she gained crucial experience needed to start her own company. Rachel founded Neurogene in 2018, a company whose mission is to harness the power of genetic medicine to improve the lives of patients and families, like hers, who are affected by rare neurological conditions.

Neurogene reached a significant milestone in 2021 when it received FDA clearance to begin the company's first clinical trial. This first-in-human clinical trial of an investigational gene therapy is for CLN5 Batten disease, a rare, rapidly progressing disease of the nervous system.

How does Neurogene work with the patient community?

Here at Neurogene, we consider patients and families to be experts. Their unique insights help us better understand how a rare disease truly impacts patients and their families. We listen to patients and families and incorporate their perspective throughout the research and development process. Patients and families are at the core of our work. We recognize and honor that it is their contributions that make scientific advances possible.

The Patient Advocacy and Engagement team is led by Kimberly Trant, Executive Director. Kimberly reports directly to Neurogene's CEO, Rachel McMinn, Ph.D., further demonstrating the company's dedication to ensuring the patient and family perspective is integrated into the company's work. Kimberly started her career as a registered nurse, before receiving her MBA and transitioning into multiple leadership roles in pharmaceutical and biotechnology companies over 20 years ago.

Gay Grossman, Director, Patient Advocacy and Engagement, brings valued experience to the team as a parent of a child with a rare disease. Gay started her journey in rare disease over 2 decades ago when, due to her enduring advocacy, her daughter was the first diagnosed patient worldwide with her rare condition. Gay and her husband then built a family foundation and a strong community for families with the same diagnosis.

How can Neurogene be contacted? Is Neurogene on social media?

Neurogene can be contacted by phone or email.

- By phone: 866-381-7185 (US +1)
- Patients and families can reach us at: patientinfo@neurogene.com
- Healthcare providers can reach us at: medicalinfo@neurogene.com

We are on social media at the following channels.

- Neurogene Inc. Facebook page: https://www.facebook.com/NeurogeneInc/
- Neurogene Inc. Twitter handle: https://twitter.com/NeurogeneInc/
- Neurogene Inc. LinkedIn profile: https://www.linkedin.com/company/NeurogeneInc

The entire Neurogene team is excited to advance our development program for Rett syndrome. We look forward to learning from you and integrating your perspective into our work. Please recognize that the development process takes time, and we are early in our journey together. We are still in our preclinical (non-human) research phase and are not yet conducting clinical trials in humans. There is not a clear timeline we can provide at this time; it is simply too early. We will provide updates as they become available and will collaborate with the patient advocacy organizations to share information. In the meantime, please do not hesitate to reach out if you want to share your story or get to know us better. We look forward to hearing from you!

Sincerely,

The Patient Advocacy and Engagement Team at Neurogene

Kimberly Trant, Executive Director

Gay Grossman, Director