# Neurogene (https://www.neurogene.com)

Focusing on the Future of Life-Changing Genetic Medicines

Gene therapy holds incredible promise to revolutionize medical treatment. At Neurogene we believe that innovation, robust scientific execution, and a strong sense of purpose are required to turn this promise into reality. Our focus is to develop life-changing genetic medicines for people and their families impacted by devastating neurological diseases.

## Technology

In certain diseases, we need to be EXACT

Many complex genetic diseases are not amenable to conventional gene therapy because gene expression is not adequately controlled. Neurogene is developing EXACT gene therapy technology, reimagining what is possible for complex genetic diseases.

Too little - gene expression can cause disease.

CONTROLLED GENE

EXPRESSION

Too much - gene expression can cause toxicity.

## EXACT Technology

Our Gene Therapy Platform Technology (EXACT)

Delivers highly controlled, consistent levels of transgene expression. Our lead gene therapy candidate utilizing this technology is for Rett syndrome (MECP2).

Using modular elements, EXACT technology can be tuned to enable optimized levels of transgene expression.

May be employed with any therapeutic transgene that can be packaged into adeno-associated virus (AAV) or used in other viral and nonviral delivery platform technologies.

By providing a safety valve against overexpression toxicity, EXACT has demonstrated a superior safety profile over conventional gene therapy in multiple preclinical models.

EXACT = Expression Attenuation via Construct Tuning

## Robust Internal Manufacturing Capabilities

### *QUALITY*

Quality is at the forefront of our operations. Our CGMP-compliant processes have been designed to reduce product-and process-related impurities, and we strive to continually optimize our gene therapy products.

### *PROCESS DEVELOPMENT*

We have developed two independent robust and scalable platform processes to support production of research and clinical grade material using either HEK293 (mammalian) or Sf9 (insect) cells in suspension.

### *ANALYTICAL DEVELOPMENT*

We have developed state of the art methodologies and collaborate with third parties to support robust characterization of our gene therapy products.

### *MULTI-PRODUCT FACILITY*

We employ single use technologies to allow multiple products to be produced at our 42,000 square foot facility.

## About Neurogene

At Neurogene, our focus is to develop life-changing genetic medicines for patients and their families affected by rare, devastating neurological diseases.

### OUR VISION

Push the boundaries of genetic medicine to address complex and devastating neurological diseases.

### OUR MISSION

Turn devastating neurological diseases into treatable conditions, to improve the lives of patients and families impacted by these rare diseases.

### OUR CULTURE

Build a corporate culture that nurtures innovation, creative problem solving, and a strong sense of purpose, while honoring the patient/caregiver mindset.

## Our Core Values

### *IT'S BETTER TOGETHER*

We address the incredible complexity of gene therapy as a team, gathering insights, ideas, and perspectives from our internal and external subject matter experts and stakeholders.

### *KEEP AN OPEN MIND*

In the rapidly evolving field of gene therapy, we regularly ask ourselves what we can do differently to solve emerging challenges.

### *PATIENTS AND FAMILIES ARE WAITING*

Knowing that families urgently await treatment options, we work with a sense of urgency balanced with the necessary scientific rigor to advance our gene therapy programs.

### *REIMAGINE THE FUTURE*

We prize innovation, encourage new ideas, and actualize our vision to drive transformation in genetic medicine.

## Our Leadership Team

Neurogene's senior leadership team brings a strong sense of purpose and extensive drug development experience to the task. Our founding leaders include:

Rachel McMinn, Ph.D.

Founder and Chief Executive Officer

Christine Mikail, J.D.

President and Chief Financial Officer

Stuart Cobb, Ph.D.

Chief Scientific Officer

Albena Patroneva, M.D., MBA

Senior Vice President, Clinical Development

Effie Albanis, M.D.

Senior Vice President, Early Clinical and Translational Research

Andrew Mulberg M.D.

Senior Vice President, Regulatory Affairs, Quality Assurance, and Quality Control

Ricardo Jimenez

Senior Vice President, Technical Operations

Arvind Sreedharan

Senior Vice President, Business Operations

## Board of Directors

Rachel McMinn, Ph.D.

Chief Executive Officer

Robert Baffi, Ph.D.

Venture Partner

Cory Freedland, Ph.D.

Venture Partner

Keith Woods

Advisor

Rohan Palekar

Chief Executive Officer

Sarah B. Noonberg, M.D., Ph.D.

Chief Medical Officer

## Pipeline

| Product Candidate | Indication | IND* Enabling | Phase I/II | Pivotal | Near-Term Expected Milestones |
|---|---|---|---|---|---|
| NGN-401 | Rett Syndrome | Transgene Regulation | | | Preliminary Data 4Q'24<br>Additional Data 2H'25 |
| NGN-101 | CLN5 Batten Disease | CNS + Ocular Delivery | | | Preliminary Data 2H'24 |

*IND = Investigational New Drug

## Partnering with the University of Edinburgh

Neurogene has a research collaboration with the University of Edinburgh, a world leader in biomedical and translational research for neurodevelopmental diseases.

The collaboration provides comprehensive research capabilities to Neurogene, enabling the company and the University to accelerate scientific innovation to continue to improve upon conventional gene therapy.

(https://edinburgh-innovations.ed.ac.uk/case-studies/neurogene)

## Clinical Trials

### *A Novel, Regulated Gene Therapy (NGN-401) Study for Female Children With Rett Syndrome*

Gene therapy clinical trials are research studies that help determine whether a gene therapy is safe and effective to treat a specific genetic disease.

(https://www.clinicaltrials.gov/study/NCT05898620?cond=Rett%20Syndrome&rank=2)

### *Gene Therapy Study for Children With CLN5 Batten Disease*

(CLN5-200)

Gene therapy clinical trials are research studies that help determine whether a gene therapy is safe and effective to treat a specific genetic disease

Investigational Gene Therapy

Clinical Trial

for Children with

CLN5 Batten Disease

Enrolling Now

For more information call:

+1 (877) 237-5020

patientinfo@neurogene.com

Clinical Trial information can be found on

www.ClinicalTrials.gov search #NCT05228145

About the Clinical Trial

What is the title of the gene therapy trial?

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 Subtype 5 (CLN5) Disease. This gene therapy clinical trial is a first of its kind, designed to address the neurodegeneration and vision loss associated with CLN5 Batten disease.

What is the purpose of the clinical trial?

The purpose of this gene therapy clinical trial is to assess the safety and potential for efficacy of an investigational gene therapy in children 3-9 years old with genetically confirmed Neuronal Ceroid Lipofuscinosis subtype 5 (CLN5) Batten disease.

Clinical Trial FAQ

What type of clinical trial is this?

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.

Is there an age requirement for participation in the trial?

Yes, the gene therapy clinical trial will enroll children who are 3 to 9 years old, with a genetic diagnosis of Batten disease subtype CLN5.

What is the route of administration into the body?

The investigational gene therapy 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.

How long does the clinical trial last?

Each participant will be followed for safety and efficacy for 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.

Is there a cost to participate?

Trial-related costs and expenses are paid or reimbursed by the clinical trial sponsor, Neurogene. There is a comprehensive travel and expense policy in place for costs and expenses related to the clinical trial. More details of the specific policy can be provided by the clinical trial site.

Is there someone to help with travel arrangements?

Yes, travel and accommodations will be arranged for participants in the clinical trial. The clinical trial staff will provide more details.

Are patients from all geographic areas eligible to enroll in this trial?

Although the clinical trial sites are in the US and the UK, patients from around the world will be considered for enrollment and are encouraged to contact Neurogene for more information +1 (877) 237-5020. Professional translators will be available if needed.

Study Locations

This clinical trial is now enrolling patients at:

University of Rochester Medical Center in Rochester, NY, USA.

Great Ormond Street Hospital, London, United Kingdom

Patients from around the world will be considered for enrollment and are encouraged to contact Neurogene for more information +1 (877) 237-5020.

Contact

Families are encouraged to contact Neurogene at:

+1 (877) 237-5020

patientinfo@neurogene.com

Healthcare providers may contact Neurogene Inc.

+1 (877) 237-5020

medicalinfo@neurogene.com

### *A Natural History Study of Neuronal Ceroid Lipofuscinosis Type 5 (CLN5)*

A natural history study is an essential component of developing and expediting treatments, where the natural history is not well documented. Participating in a natural history study enables a rigorous scientific evaluation of disease characteristics and disease progression, which provides essential information to design a clinical trial that allows for the evaluation of whether a treatment is safe and effective.

(https://www.clinicaltrials.gov/study/NCT03822650)

## General Contact

+1 (877) 237-5020

medicalinfo@neurogene.com

## Expanded Access Policy

Neurogene is focused on developing life-changing genetic medicines for patients and their families affected by rare, devastating neurological diseases. To accomplish this, we collaborate with many researchers, the patient community, patient advocacy organizations, and physicians, in addition to working closely with global regulatory agencies. With the development of medicines that will be safe,

effective, and satisfy unmet medical need, comes the promise that these medicines will be broadly accessible to appropriate patients in the future.

### *Our Policy*

Currently, Neurogene does not offer an expanded access program.

Neurogene carefully considered our policy on providing access to investigational gene therapies outside of a clinical trial. We took into account multiple considerations, including patient safety, potential risks and benefits, the impact to the broader patient population, resourcing, and other ethical considerations. At this time in development, availability and access to Neurogene investigational therapies is possible only through participation in clinical trials. Clinical trials are carefully regulated, monitored, and conducted in specific patient populations to assess safety, and serve a critical role in understanding the risks and potential benefits of investigational therapeutic options, such as gene therapy. For these reasons, we believe that completing the necessary clinical trials and obtaining the data needed for review and approval by regulatory agencies is the fastest and most scientifically sound way toward making Neurogene's gene therapies available to the broader patient population.

If you are a patient or family member and have questions about this policy, please contact PatientAdvocacy@neurogene.com. If you are interested in learning more about our current investigational gene therapy programs, visit pipeline (https://www.neurogene.com/pipeline/) or our Patients and Families page (https://www.neurogene.com/patients-and-families/).

If you are a healthcare provider and have questions about this policy, please contact medicalinfo@neurogene.com.

## Patients and families

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 disease.

### Why I Founded Neurogene

Rachel McMinn, Ph.D.

Founder and Chief Executive Officer

"My older brother lives with an undiagnosed rare neurological disease. I have witnessed firsthand, initially as a sibling and then into my adult life, the devastating impact his condition has had on my entire family. His condition inspired a profound sense of purpose and responsibility in me to want to make the world a better place for other people and families living with similar challenges.

I dedicated my early life to science, studying chemistry in college, completing a Ph.D. in chemistry and molecular biology, and accepting a post-doctoral fellowship in cell and molecular biology. I then spent the next 13 years as a Wall Street biotechnology equity analyst, learning about cutting edge science, its application to develop transformative medicines, and the recipes for company failures and successes.

I left my Wall Street career in order to learn how to directly apply my scientific and analytical skills in advancing treatments to patients. I took on the role of Chief Business and Strategy Officer at Intercept Pharmaceuticals, and spent several years gaining leadership and operational experience. However,

when gene therapy began to show that life-altering improvements for patients with genetic diseases was possible, it was a crystalizing moment when I knew I needed to be part of this genetic medicine revolution, and it provided the catalyst for me to found Neurogene in 2018."

## Patient Advocacy & Engagement at Neurogene

Kimberly Trant

Executive Director

Patient Advocacy & Engagement

+1 (877) 237-5020

patientinfo@neurogene.com

## Our Approach

A foundational principle of our work is to integrate the perspective of the rare patient community into everything we do.

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. We strive to support them throughout their participation in clinical research.

## Clinical Programs and Resources

Neurogene's commitment to patients and families affected by severe neurological diseases goes beyond the science; we want to raise awareness and connect the rare disease communities to resources and support that can help guide their journey.

## Additional information and resources

### *BATTEN DISEASE*

Batten disease, also referred to as neuronal ceroid lipofuscinoses, or NCLs, is a group of rare, inherited diseases of the nervous system. There are different subtypes of Batten disease caused by dysfunction in one of 13 different genes. Batten disease subtypes share many common features but differ in terms of age of onset and rate of progression. Batten disease has an estimated incidence of 2 to 4 out of every 100,000.

Neurogene's lead Batten disease program is a gene therapy for the treatment of CLN5 Batten disease, a rapidly progressive neurodegenerative disease which primarily manifests during early childhood, and results in early mortality. CLN5 is caused by a mutation in the CLN5 gene that leads to loss of normal CLN5 protein function, including various aspects of lysosomal function, lipid metabolism, and neuronal viability.

Children with CLN5 typically experience developmental delay followed by a steep period of regression of previously acquired skills, including loss of gross and fine motor skills, loss of speech, loss of vision,

development of recurrent seizures, and a progressive deterioration in intellectual function and cognition. Ultimately, the disorder leads to a significantly reduced quality of life for both the patient and family.

Currently, there are no approved disease modifying therapies available, and treatment for CLN5 Batten disease includes symptom management and supportive care. Neurogene's CLN5 program is currently in the early clinical trial stage.

### Clinical Trials

#### Gene Therapy Study for Children With CLN5 Batten Disease (CLN5-200)

Gene therapy clinical trials are research studies that help determine whether a gene therapy approach is safe for people. They also help uncover the effects of gene therapy on the body.

(https://www.neurogene.com/patients-and-families/about-batten-disease/cln5-batten-disease-clinical-trial/)

#### A Natural History Study of Neuronal Ceroid Lipofuscinosis Type 5 (CLN5)

A natural history study is an essential component of developing and expediting treatments, where the natural history is not well documented. Participating in a natural history study enables a rigorous scientific evaluation of disease characteristics and disease progression, which provides essential information to design a clinical trial that allows for the evaluation of whether a treatment is safe and effective.

(https://www.clinicaltrials.gov/study/NCT03822650)

### Contact

+1 (877) 237-5020

patientinfo@neurogene.com

### Resources

Living Batten Disease Awareness Website

Discover helpful resources and support for CLN5 Batten disease, a rare neurological condition.

(https://www.livingbatten.com/)

Learn more about Batten disease and Gene Therapy

Batten disease occurs when there is a mutation (variant) in one of the thirteen known neuronal ceroid lipofuscinosis (NCL) genes. Gene therapy may offer a one-time treatment to slow or ideally stop the progression of these conditions by delivering a functional gene, which addresses the root cause of the condition. Watch this video from the American Society of Gene & Cell Therapy.

Video (https://www.youtube.com/watch?v=5_VdzpOa0zw)

### *RETT SYNDROME*

Rett syndrome is a rare genetic disorder that occurs almost exclusively in females, and leads to severe impairments that affect nearly every aspect of their lives. This includes their ability to speak, walk, eat, and breathe. The impact on patients and families is profound. Most females are nonverbal, lack motor

skills, and require 24-hour care for all activities of daily living. Many females with Rett syndrome appear to understand the world around them, leaving many caregivers feeling that their daughters are intellectually and emotionally intact.

Rett syndrome is an X-linked, progressive, neurodevelopmental disorder. It has an estimated worldwide incidence of 1 out of every 10,000-15,000 live female births.

The incidence in males is currently unknown. Advances in genetic testing and phenotypic identification have revealed that MECP2 mutations in males are responsible for a wide spectrum of neurological disorders, including Rett syndrome.

Rett syndrome is caused by mutations in the MECP2 gene that lead to deficiency of the methyl cytosine binding protein 2, an important protein responsible for normal function in the brain and other parts of the nervous system.

Females with Rett syndrome typically have normal development until 6-18 months of age, followed by a progressive deterioration of acquired skills such as gross and fine motor skills, purposeful hand function and communication. They subsequently develop stereotypic hand movements such as hand-wringing.

Over time females may develop muscle contractures, rigidity, and debilitating scoliosis, along with periods of recurrent seizures, and burdensome gastrointestinal and breathing abnormalities.

Although there are treatments available for Rett syndrome, there is no treatment option that addresses the root cause of disease and a significant unmet need still exists for new treatment options.

## Resources

Rett Syndrome Research Trust

The Rett Syndrome Research Trust has a singular mission: a cure for Rett syndrome. Our remarkable progress is made possible by the support, passion, and commitment of Rett families around the world.

(https://reverserett.org/)

International Rett Syndrome Foundation

To accelerate full spectrum research to cure Rett syndrome and empower families with information, knowledge and connectivity.

(https://www.rettsyndrome.org/)

Reverse Rett

Reverse Rett is a patient advocacy and research organisation focused on delivering treatments and a cure for Rett syndrome to everyone affected.

(https://www.reverserett.org.uk/)

## Follow Us on Social Media

Connecting families with the latest Neurogene content and specific resources related to CLN5 Batten disease.

Follow Our Neurogene Facebook Page (https://www.facebook.com/NeurogeneInc/)

Follow Our Living Batten Facebook Page (https://www.facebook.com/LivingBatten/)

## Additional Resources

(https://www.neurogene.com/additional-resources/)

Additional Resources

### *The National Organization for Rare Disorders*

The National Organization for Rare Disorders (NORD®), an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families. We work together to accelerate research, raise awareness, provide valuable information and drive public policy that benefits the estimated 25-30 million Americans impacted by rare diseases.

www.rarediseases.org

### *Global Genes*

Global Genes is an organization that helps to build awareness, educate the global community, and provide critical connections and resources that equip advocates to become activists for their rare diseases.

www.globalgenes.org

### *Child Neurology Foundation*

The Child Neurology Foundation connects partners from all areas of the child neurology community so those navigating the journey of disease diagnosis, management, and care have the ongoing support of those dedicated to treatments and cures.

https://www.childneurologyfoundation.org/

### *EveryLife Foundation*

EveryLife Foundation is dedicated to advancing the development of treatment and diagnostic opportunities for rare disease patients through science-driven public policy.

www.everylifefoundation.org

### *Eurordis*

EURORDIS-Rare Diseases Europe is a not-for- profit alliance of 1000 rare disease patient organisations from over 70 countries around the world that work together to improve the lives of the 30 million Europeans living with a rare disease. By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

https://www.eurordis.org/

### *Rare Disease Network*

Rare Diseases Clinical Research Network is a group committed to advancing medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment, and data sharing.

https://www.rarediseasesnetwork.org/

### *Genetic Alliance*

Genetic Alliance is an advocacy organization that engages individuals, families, and communities to transform health.

www.geneticalliance.org

### *Alliance for Regenerative Medicine*

The Alliance for Regenerative Medicine is an international community of small and large companies, non-profit research institutions, patient organizations, and other sector stakeholders dedicated to realizing the promise of regenerative medicine for patients around the world.

https://alliancerm.org/

### *ARM Foundation*

The ARM Foundation for Cell and Gene Medicine educates, engages, and empowers patients, caregivers, industry leaders, and other stakeholders to help advance the science and benefits of gene and cell therapy.

www.thearmfoundation.org

### *American Society of Gene and Cell Therapy (ASGCT)*

The American Society of Gene and Cell Therapy (ASGCT) is a professional membership organization for scientists, physicians, advocates, and other professionals in gene and cell therapy. A goal of the Society is to provide timely, accurate, and responsible information about gene and cell therapy to patients and their families through the Patient Education website, including resources on gene therapy for Batten disease.

https://patienteducation.asgct.org/disease-treatments/batten

### *National Alliance for Caregiving*

Established in 1996, the National Alliance for Caregiving is a non-profit coalition of national organizations focusing on advancing family caregiving through research, innovation, and advocacy. The Alliance conducts research, does policy analysis, develops national best-practice programs, and works to increase public awareness of family caregiving issues. Recognizing that family caregivers provide important societal and financial contributions toward maintaining the well-being of those they care for, the Alliance supports a network of more than 80 state and local caregiving coalitions and serves as Founder and Secretariat for the International Alliance of Carer Organizations (IACO).

https://www.caregiving.org/

### *Courageous Parents Network's (CPN)*

Courageous Parents Network's (CPN) mission is to empower, support and equip families and providers caring for children with serious illness. CPN's vision promotes the family's journey as one in which they have confidence in their ability to be the best caregivers they can possibly be, resulting in minimal regret and maximal healing. CPN resources include 500+ original short videos, a blog, curated content modules (Guided Pathways) and downloadable guides on topics spanning the wide range of issues including coping with the diagnosis, understanding anticipatory grief, working with the medical team, making difficult decisions about medical interventions and coping following loss. Much of this content is also offered in Spanish.

https://courageousparentsnetwork.org/

### *The Mighty*

The Mighty is a supportive health community for people facing challenges and their families.

www.themighty.com

## Careers

Join a Team That is Making an Impact

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 disease.

We are building a team of passionate, driven individuals who want to join us on our mission and be part of a culture with a strong sense of purpose. To apply, please submit your resume and cover letter to careers@neurogene.com.

Neurogene is an Equal Opportunity Employer and will assess all applicants based on their qualifications for the job, without regard to race, color, sex, religion, national origin, age, disability, sexual orientation, gender identity, protected veteran, disability status or any other characteristics protected by applicable federal, state or local law.

## Contact Us

Developing genetic medicines for rare neurological diseases to improve the lives of patients and families around the world

### Neurogene's CORPORATE HEADQUARTERS

535 W 24th Street, 5th Floor

New York, NY 10011

### For MEDICAL INQUIRIES

+1 (877) 237-5020

medicalinfo@neurogene.com

### For PATIENT AND FAMILY INQUIRIES

+1 (877) 237-5020

patientinfo@neurogene.com

### For INVESTOR AND MEDIA INQUIRIES

neurogene@argotpartners.com

### For OTHER INQUIRIES

+1 (877) 237-5020

info@neurogene.com

## Privacy

This Privacy Notice ("Privacy Notice") was last updated and effective on December 18, 2023.

Neurogene Inc. ("Neurogene") respects the privacy of all individuals who entrust us with their personal data (i.e., information from or about an identified or identifiable person, including information that we can associate with an individual person). This Privacy Notice explains what types of personal data Neurogene may collect from you, how we collect it, how we use it, who we may disclose it to, and how you can manage it. It also describes the policies and practices that we have developed to safeguard personal data and to comply with applicable data protection laws. Please read this Privacy Notice and our Terms of Use carefully.

Our Privacy Notice is not a contract, and it does not create any legal rights or obligations. We may amend this Privacy Notice at any time. When this Privacy Notice is changed, the date of the latest revision will appear at the top of this page.

If you have any questions regarding Neurogene's privacy practices or wish to access or correct personal data Neurogene has collected from you, please contact us using the information below:

Company name: Neurogene Inc.

Mailing address: 535 West 24th Street, 5th Floor New York, NY 10011

Person responsible for privacy inquiries: Carleen D. Lyken

Senior Counsel

Email address: Privacy@neurogene.com

## Information We May Collect About You

We may collect, use, store and transfer different categories of personal data about you in electronic (e.g., email, photographs, documents) or paper media. We have grouped them together as follows:

Identity Data includes first name, maiden name, last name, username or similar identifier, marital status, and title.

Contact Data includes address, email address and telephone numbers.

Transaction Data includes details about payments to and from you and other details of products and services you have purchased from us.

Technical Data includes internet protocol (IP) address, your login data, browser type and version, time zone setting and location, browser plug-in types and versions, operating system and platform and other technology on the devices you use to access our Website or intranet.

Usage Data includes information about how you use our Website, intranet, products and services, including: the domain name of the website that allowed you to navigate to our Website, search engines used, the length of time spent on our Website, the pages you looked at on our Website, the frequency of your visits to our Website, and other relevant statistics.

Professional, Employment-Related, and Candidacy Information. When you apply for a job with us, we will collect various of the information categories described above. In addition, we may collect additional identifiers, such as: Social Security Number, driver's license or state identification number, veteran status, race or ethnic origin, gender and other personal and online identifiers; your resume or CV, cover letter, previous work and education experience, and any other professional data collected as part of your employment application and our hiring process; residency, citizenship, or work permit status; and information required for us to comply with laws, including at the direction of law enforcement authorities or court orders.

Surveys. We may contact you to participate in surveys. If you decide to participate, you may

be asked to provide certain information which may include personal data.

Interactive Features. We and others who access our Website may collect personal data that you submit or make available through our interactive features (e.g., social media pages). Any information you provide on the public sections of these features will be considered "public", unless otherwise required by applicable law, and is not subject to the privacy protections referenced herein.

Additional Information. Additional information that you provide to us, including through feedback, messages, emails, mail, or otherwise.

## How We Collect Information About You

Direct interactions. You may give us your Identity Data and Contact by filling in forms or by corresponding with us by mail, phone, and e-mail or otherwise. This includes personal data you provide when you contact us through the Website, apply for employment with us.

Automated interactions. As you interact with our Website or intranet, we may automatically collect Technical Data and Usage Data about your equipment, browsing actions and patterns, subject to your consent where required. We collect this personal data by using cookies, and other similar technologies. For additional information about how Neurogene uses cookies and similar technologies, see "Cookies and Similar Technologies" section.

Third Parties (or publicly available sources). We may receive categories of personal data about you from various third parties and public sources as set out below:

Technical Data from analytics providers such as Google and search information providers.

Identity and Contact Data from recruitment agencies.

## How We Use Your Information

Neurogene does not disclose, give, or sell any personal data you provide to any outside organizations for any reason (other than as described below). We may use any information we collect about you or about your use of the Website for the following purposes:

Provide Our Website. In connection with the operation of our business, as well as to improve the Website or to communicate with you about our business or otherwise in connection with our management of the Website. We may also use information we collect about you for security and protection of personnel, assets, and resources; regulatory compliance and monitoring; and compliance with legal requirements or to defend or pursue legal claims.

Website Administration and Development. We may also monitor traffic patterns and Website usage to maintain, protect, and improve our Website, ensure the technical functions of Our network, and help us develop the design and layout of the Website. We use application logs on your device and our server as well as "cookies" and other tracking technologies for the purposes described below as well as to enhance the functionality of the Website. This information may be stored in files on your device that we access.

Changes to Website. We may also use the information we collect to occasionally notify you about functionality changes to the Website.

Evaluate Your Candidacy for a Job with Us. As a job applicant or recruit, we may use your personal data to evaluate your candidacy for a position with us.

Legal Reasons. To comply with applicable law, respond to valid legal process, participate in legal proceedings, including civil discovery and litigation, protect you, us, and others from unlawful or fraudulent activities, and investigate potential violations of and enforce our policies, including our Terms of Use.

With Your Consent. We may otherwise use your personal data with your consent.

## How We Share Your Information

We may share the personal data we collect about you as described below:

Service Providers. We may employ independent contractors, consultants, vendors, and suppliers, such as third-party service providers, call centers, mail houses, or any other third party who may need to receive or handle your personal data on our behalf (collectively, "Outside Contractors") in connection with the performance of obligations under this Privacy Notice and to provide specific services and products related to the Website and our business. In the course of providing products or services to us, these Outside Contractors may sometimes have access to your personal data.

Subsidiaries and Affiliates. We may share your personal information with our subsidiaries and affiliates for the purposes described above or as reasonably necessary for our internal administrative and business purposes.

Legal Reasons. We may disclose information we have about you to regulatory authorities, law enforcement agencies, or as required by applicable law.

Change of Control Transaction. We may share personal data with a buyer or other successor in the event of a merger, divestiture, restructuring, reorganization, dissolution or other sale or transfer of some or all of the Company's assets, whether as a going concern or as part of bankruptcy, liquidation or similar proceeding, in which personal data held by the Company about individuals who access our Website is among the assets transferred.

With Your Consent. We may otherwise share your personal information with your consent.

## How We Protect Your Information

We have put in place appropriate security measures to prevent your personal data from being accidentally lost, used or accessed in an unauthorized way, altered or disclosed. In addition, we limit access to your personal data to those employees, agents, contractors and other third parties who have a business need to know. They will only process your personal data on our instructions and they are subject to a duty of confidentiality.

We have put in place procedures to deal with any suspected personal data breach and will notify you and any applicable regulator of a breach where we are legally required to do so.

## Cookies and Similar Technologies

Cookies are small files that many websites place on your hard drive that allow those websites to identify you. For example, if you allow a website to remember your login name or password, that website places a cookie on your computer.

We use cookies for technical purposes, statistical purposes and advertising purposes. Indeed, subject to your prior consent, we may place cookies on your computer to allow us to identify you during future visits to our Website. We may use cookies to measure web traffic and to customize your visit. If you consented to our use of cookies but later wish to opt out, you can change the browser settings, at any time, in order to block the cookies. If you block cookies, you may not be able to use certain features or functions of this Website, or this Website may not operate in optimal mode.

A beacon is an electronic tracking mechanism that usually consists of a single-pixel image. It can be embedded in a web page or in an email to transmit information, which could include personal data. For example, it allows an email sender to determine whether a particular email has been opened.

We may also use Google Analytics or other similar software or services, with your prior consent, to gather certain information in connection with the use of the Website. When we use Google Analytics, your web browser automatically sends certain information to Google about your use of the Website, such as the web address of the page you're visiting and your IP address. You may opt-out by visiting this link. For more information on how Google Analytics collects, protects, uses, and shares your data, click here.

## How We Respond to Do Not Track Signals

Some web browsers may transmit "do-not-track" signals to websites with which the browser communicates. Websites linked to this Privacy Notice do not currently respond to these "do-not-track" signals.

## Choices You Have About Our Use of Your Information

You can write to us at any time to obtain a copy of your information and to have any inaccuracies corrected or if you no longer wish to be registered on the Website. Where appropriate, you may have your personal data erased, rectified, amended, or completed. In order to contact us regarding your information, you may send us an email at privacy@neurogene.com.

## Links to Third Party Websites

The Website may contain links to other websites. These third-party sites may send their own cookies to you to collect data or solicit personal data. Please be aware that other sites are not subject to this Privacy Notice and Neurogene is not responsible for the privacy practices of these other sites. We encourage you to be aware when you leave our site and to read the privacy statements of each and every website that collects personal data. This Privacy Notice applies solely to information collected by the Website.

## Children's Privacy

The Website is not directed to children under the age of 13. We do not knowingly collect personal data from children under the age of 13. If an individual identifies themselves as a child under the age of 13, we will not collect, store, or use any personal data. If we receive personal data that we discover was provided by a child under the age of 13, we will promptly destroy such information.

## Updating this Privacy Notice

From time to time, we may change our privacy practices.

We will post any updates to this Privacy Notice on our Website, with a "last updated and effective" date at the top of this document. Please check this page for updates.

## SUPPLEMENTAL UK PRIVACY NOTICE

For purposes of United Kingdom ("UK") data protection laws, Neurogene Inc. is the data controller, i.e., the company responsible for controlling the processing of personal data covered by this Privacy Notice.

The UK Data Protection Act 2018 (the "DPA 2018") and the UK GDPR (as defined in section 3(10) (as supplemented by section 205(4)) of the DPA 2018) require Neurogene as the data controller to provide additional and different information about its data processing practices to data subjects in the UK. If you are a data subject within the UK, this Supplemental UK Privacy Notice applies to you in addition to the provisions above.

## HOW WE USE YOUR PERSONAL DATA

We will only use your personal data when the law allows us to do so. Most commonly, we will use your personal data in the following circumstances:

We need to perform the contract we are about to enter into or have entered into with you.

It is necessary for our legitimate interests (or those of a third party) and your interests and fundamental rights do not override those interests.

We need to comply with a legal or regulatory obligation.

We may also rely on consent as a legal basis for processing your personal data in certain circumstances, like sending direct marketing communications to you via email or text message. You have the right to withdraw consent at any time by contacting us.

We have set out below, in a table format, a description of many of the ways we plan to use your personal data, and which of the legal bases we rely on to do so. We have also identified what our legitimate interests are where appropriate.

Note that we may process your personal data for a different lawful basis for each purpose for which we are using your data. Please contact us if you need additional details about the specific legal ground we are relying on to process your personal data.

| **Purpose/Activity** | **Category of personal data** | **Lawful basis for processing including basis of legitimate interest** |
|---|---|---|
| To register you as a new customer, contractor, candidate or employee. | (a) Identity (b) Contact | Performance of a contract with you or to take steps prior to entering into a contract with you |
| To manage our relationship with you which will include: (a) Notifying you about changes to our Terms of Use or Privacy Notice (b) Asking you to provide feedback or take a survey (c) As an employee | (a) Identity (b) Contact (c) Profile (d) Usage (e) Marketing and Communications (f) Financial | (a) Performance of a contract with you (b) Necessary to comply with a legal obligation (c) Necessary for our legitimate interests to keep our records updated; to study how customers use our products/services; and to administer our relationships |
| To administer and protect our business and our intranet and website (including troubleshooting, data analysis, testing, system maintenance, support, reporting and hosting of data) | (a) Identity (b) Contact (c) Profile (d) Technical (e) Usage | (a) Necessary for our legitimate interests for running our business and employee relationships; provision of administration and IT services, network security; to prevent fraud; and in the context of a business reorganization or group restructuring exercise. (b) Necessary to comply with a legal obligation |
| To deliver relevant website content and advertisements to you and measure or understand the effectiveness of the advertising we serve to you | (a) Identity (b) Contact (c) Profile (d) Usage (e) Marketing and Communications (f) Technical | Consent. |
| To use data analytics to improve our website, products/services, marketing, customer relationships and experiences | (a) Technical (b) Usage | Consent. |

| | | |
|---|---|---|
| To make suggestions and recommendations to you about goods or services that may be of interest to you | (a) Identity (b) Contact (c) Technical (d) Usage (e) Profile | Consent. |
| To comply with legal requirements and to defend or pursue legal claims. | (a) Identity (b) Contact (c) Technical (d) Usage (e) Profile | Necessary for our legitimate interests Necessary to comply with a legal obligation |

## CHANGE OF PURPOSE

We will only use your personal data for the purposes for which we collected it, unless we reasonably consider that we need to use it for another reason and that reason is compatible with the original purpose.

If we need to use your personal data for an unrelated purpose, we will notify you and we will explain the new legal basis. If such change of purpose requires your consent, you will have the choice to consent as to whether or not we may use your personal data in a different manner.

Please note that we may process your personal data without your knowledge or consent, in compliance with the above rules, where this is required or permitted by law.

## INTERNATIONAL TRANSFERS OF PERSONAL DATA

We are based outside the UK, so the processing of your personal data may involve a transfer of data outside the UK. Information on how to contact our privacy officer can be found at the introduction of this Notice.

Whenever we transfer your personal data out of the UK, we ensure a similar degree of protection is afforded to it by ensuring at least one of the following safeguards is implemented:

We will only transfer your personal data to countries that have been deemed to provide an adequate level of protection for personal data by the UK Secretary of State.

Regarding transfers to a country whose legislation has not been recognized by the UK Secretary of State as having an adequate level of protection (for instance, transfers to the US), we are required to incorporate the International Data Transfer Agreement in agreements in order to provide similar protection to personal data shared within the UK. In this regard, please note that we will provide you with a copy of applicable safeguards upon request, at the contact details specified at the end of this Notice.

## HOW LONG WE RETAIN YOUR PERSONAL DATA

We will only retain your personal data for as long as necessary to fulfil the purposes we collected it for, including for the purposes of satisfying any legal, accounting, or reporting requirements.

To determine the appropriate retention period for personal data, we consider the amount, nature, and sensitivity of the personal data, the potential risk of harm from unauthorized use or disclosure of your personal data, the purposes for which we process your personal data and whether we can achieve those purposes through other means, and the applicable legal requirements.

With respect to the services and products provided to you, we will keep your personal data during the period of our contractual relationship extended by the applicable limitation period. With regard to marketing communications with prospects, we will keep your personal data for three (3) years after the last correspondence with us.

With respect to the processing carried out in order to protect or defend your and our rights, property and security, we will keep your personal data for the time of the relevant dispute or statute limitation period.

As to the processing carried out in order to comply with a legal requirement, we will keep your personal data for the duration of such obligation.

## YOUR DATA PROTECTION RIGHTS

Under certain circumstances, visitors from within the UK have the following data protection rights:

access to your personal data.

correction of your personal data.

erasure of your personal data.

object to processing of your personal data.

restrict of processing your personal data.

transfer of your personal data (data portability).

withdraw consent to any consent that you have previously given.

If you wish to exercise any of the rights set out above, please contact our privacy officer. You can also contact the Information Commissioner's Office at this link to make a complaint.

VeraSafe has been appointed as Neurogene's representative in the UK for data protection matters, pursuant to Article 27 of the UK GDPR. VeraSafe can be contacted in addition to or instead of Neurogene's privacy officer, only on matters related to the processing of personal data.

To make such an inquiry, please contact VeraSafe using this contact form: https://verasafe.com/public-resources/contact-data-protection-representative or via telephone at: +44 (20) 4532 2003.

Alternatively, VeraSafe can be contacted at:

VeraSafe United Kingdom Ltd. 37 Albert Embankment London SE1 7TL United Kingdom.

You will not have to pay a fee to access your personal data (or to exercise any of the other rights). However, we may charge a reasonable fee or refuse to comply with your request if it is clearly unfounded, repetitive or excessive.

We may need to request specific information from you to help us confirm your identity and ensure your right to access your personal data (or to exercise any of your other rights). This is a security measure to ensure that personal data is not disclosed to any person who has no right to receive it. We may also contact you to ask you for further information in relation to your request to speed up our response.

We try to respond to all legitimate requests within one month. Occasionally it may take us longer than a month if your request is particularly complex or you have made a number of requests. In this case, we will notify you and keep you updated.

## Terms of Use

These Terms of Use were last revised on June 18, 2019.

Please Read These Terms of Use Carefully Before Using This Website.

These Terms of Use ("Terms") describe the terms and conditions applicable to your use of the Neurogene Inc. ("Neurogene") website (the "Website"). By using this Website, you represent you are at least 18 years of age and you agree to be bound by and to comply with these Terms. If you do not agree to all of these Terms, do not use this Website. These Terms may periodically change without notice, so you should check these Terms before every attempt to use this Website. When these Terms are changed, the date of the latest revision will appear at the top of this page. Neurogene reserves the right at any time and without notice to change this Website. If you continue to use the Website, you signify your agreement to any revisions to these Terms.

## Privacy Notice

On this Website Neurogene may collect personal information about you. Please refer to Neurogene's Privacy Notice ("Privacy Notice") for details about how we handle your personal information.

## Intellectual Property Rights

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## Third Party Content and Linking Policy

This Website may contain links to websites operated by other parties, as well as articles, photographs, text, graphics, pictures, designs, sound, video, information, and other content or items belonging to or originating from third parties ("Third Party Content"). Such Third-party Content is not under the control of Neurogene, and Neurogene is not responsible for the content available on any other Internet sites linked to this Website. Such Third-party Content does not imply Neurogene's endorsement of material on any other site and Neurogene disclaims all liability with regard to your access to such Third-party Content. Should you decide to leave this Website and access or use any Third-party Content, you do so at your own risk and you should be aware that Neurogene's terms and policies no longer govern. You should review the applicable terms and policies, including privacy and data gathering practices, of any site to which you navigate from this Website.

You must adhere to Neurogene's linking policy as follows: (i) any link to this Website must be a text-only link clearly marked "Neurogene.com" or "Neurogene website;" (ii) the appearance, position and other aspects of the link may not be such as to damage or dilute the goodwill associated with Neurogene's names and trademarks; (iii) the link most "point" to neurogenestage.wpengine.com and not to other pages within the Website; (iv) the appearance, position and other attributes of the link may not create the false appearance that your organization or entity is sponsored by, affiliated with, or associated with Neurogene; (v) when selected by a user, the link must display the Website on full-screen and not within a "frame" on the linking website; and (vi) Neurogene reserves the right to revoke its consent to the link at any time and in its sole discretion.

## Disclaimers

This Website may be unavailable from time to time for maintenance or other reasons. Neurogene shall have no responsibility for any interruption, delay in operation or transmission, theft or destruction of, unauthorized access to, or alteration of, any content made available via the Website.

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## Limitation of Liability

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GOODWILL, BUSINESS OPPORTUNITY OR ANTICIPATED SAVINGS OR LOST DATA, OR FOR ANY DIRECT, INDIRECT, INCIDENTAL, CONSEQUENTIAL, OR SPECIAL DAMAGES, WHETHER BASED IN CONTRACT, TORT, STRICT LIABILITY OR OTHERWISE, ARISING OUT OF OR IN ANY WAY CONNECTED WITH ACCESS TO OR USE OF OR RELIANCE UPON THIS WEBSITE OR CONTENT ON THIS WEBSITE, OR SITES TO WHICH THIS WEBSITE IS LINKED, EVEN IF NEUROGENE HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, INCLUDING BUT NOT LIMITED TO DAMAGES THAT ARISE IN CONNECTION WITH MISTAKES OR OMISSIONS IN, OR DELAYS IN TRANSMISSION OF, INFORMATION TO OR FROM THE USER, INTERRUPTIONS IN TELECOMMUNICATIONS CONNECTIONS TO THIS WEBSITE OR VIRUSES, WORMS, TROJAN HORSES AND OTHER THREATS THAT MAY INFECT YOUR COMPUTER EQUIPMENT WHETHER CAUSED IN WHOLE OR IN PART BY NEGLIGENCE, ACTS OF GOD, TELECOMMUNICATIONS FAILURE, THEFT OR DESTRUCTION OF, OR UNAUTHORIZED ACCESS TO THIS WEBSITE, OR RELATED INFORMATION OR PROGRAMS.

### Governing Law and Severability

This Website is operated by Neurogene from its offices in New York, U.S. The law of the State of New York shall govern these Terms, without reference to its choice of law rules. If any provision of these Terms is held invalid or unenforceable by any court of competent jurisdiction, the other provisions of these Terms shall remain in full force and effect. Any provision of these Terms held invalid or unenforceable only in part or degree will remain in full force and effect to the extent not held invalid or unenforceable. Any invalid or unenforceable provision of these Terms shall be replaced with a valid and enforceable provision that will achieve, to the extent possible, the economic, business and other purposes of such invalid or unenforceable provision.

## Address

Neurogene Inc.

### Corporate Headquarters

535 W 24th Street, 5th Floor

New York, NY 10011

### Manufacturing Headquarters

Houston, TX 77025



GL-GEN-WEB-0002v8

## Phase 1/2 clinical trial for NGN-401

Neurogene has initiated a Phase 1/2 clinical trial for NGN-401, a gene therapy candidate for the treatment of Rett Syndrome. This investigational therapy is designed to deliver the full-length human MECP2 gene using Neurogene's proprietary EXACT technology. EXACT (Expression Attenuation via Construct Tuning) is a gene regulation platform that aims to deliver a controlled level of gene expression within a narrow range, addressing the issue of overexpression toxicity often associated with conventional gene therapies.

The first two pediatric patients were dosed with NGN-401 in late 2023 at Texas Children’s Hospital. The treatment has been well-tolerated so far, with no treatment-emergent or procedure-related serious

adverse events (SAEs) or observations of transgene-related overexpression. The Phase 1/2 trial plans to enroll a total of five female pediatric patients, with potential expansion based on data and health authority review.

Neurogene expects to report preliminary clinical data from the first cohort of patients in the fourth quarter of 2024, with additional data from an expanded number of patients anticipated in the second half of 2025. This timeline indicates that the company is on track to evaluate the efficacy and safety of NGN-401 in treating Rett Syndrome.

Rett Syndrome is a rare genetic disorder occurring predominantly in females, characterized by normal early development followed by a loss of acquired skills and multiple neurological symptoms. The disorder is caused by mutations in the MECP2 gene, leading to a deficiency of the methyl cytosine binding protein 2, which is vital for brain and nervous system function.

NGN-401's ICV (intracerebroventricular) delivery method is an integral part of its therapeutic strategy, targeting the treatment directly to key areas of the brain affected by Rett Syndrome. Preclinical studies of NGN-401 demonstrated significant survival benefits and improvements in Rett syndrome-like phenotypes compared to untreated control animals, with controlled MeCP2 protein levels in key brain regions.

The specific outcomes or improvements observed in the Neurogene's NGN-401 gene therapy, particularly regarding its ICV (intracerebroventricular) delivery method, are primarily based on preclinical studies and early phase clinical trials.

In the preclinical studies, NGN-401 demonstrated robust therapeutic benefits in animal models of Rett Syndrome. These benefits included:

Extended Survival: NGN-401 showed a significant survival benefit in preclinical models compared to untreated controls. This indicates that the therapy might have the potential to increase lifespan in patients with Rett Syndrome.

Improvement in Rett Syndrome-Like Phenotypes: The treatment led to improvements in symptoms and conditions that mimic those of Rett Syndrome. This suggests a potential reduction in the severity of the disease's symptoms.

Well-Controlled MeCP2 Protein Levels: NGN-401 demonstrated controlled expression levels of the MeCP2 protein in key brain regions affected by Rett Syndrome. Proper regulation of MeCP2 is crucial as both deficiency and overexpression can lead to neurological issues.

Safety and Tolerability: In preclinical models, NGN-401 was well-tolerated, and there were no signs of overexpression toxicity, a common concern with conventional gene therapies.

In the initial phase of the clinical trial, the first two pediatric patients treated with NGN-401 have so far tolerated the therapy well, with no significant treatment-emergent or procedure-related serious adverse events (SAEs). This suggests that the therapy is safe for use in humans, at least in the short term.

It is important to note that while these early results are promising, further research and clinical trials are necessary to fully understand the efficacy and safety of NGN-401 in humans. The interim efficacy data

from the ongoing clinical trial is expected to be reported in late 2024, which should provide more insight into the treatment's effectiveness.

For more detailed information

[Neurogene Inc.] (https://ir.neurogene.com/news-releases/news-release-details/neurogene-doses-first-patients-phase-12-trial-ngn-401-treatment)

[BioSpace] (https://www.biospace.com/article/releases/neurogene-announces-fda-clearance-of-ind-for-ngn-401-gene-therapy-for-children-with-rett-syndrome/)

[Rett Syndrome News] (https://rettsyndromenews.com/news/first-2-girls-dosed-clinical-trial-rett-gene-therapy-ngn-401/)

## Neurogene's clinical trial for NGN-401

In Neurogene's clinical trial for NGN-401, a gene therapy for Rett Syndrome, specific provisions are made to support families participating in the trial. These provisions are designed to alleviate the financial and logistical burdens often associated with clinical trial participation:

### Costs and Expenses

Trial-related costs and expenses are covered or reimbursed by Neurogene, the sponsor of the clinical trial. This includes a comprehensive travel and expense policy for costs related to participation in the trial.

### Travel and Accommodation Arrangements

The clinical trial staff assists with travel and accommodation arrangements for participants in the trial. This support is important for families who might be traveling from different geographic locations to participate in the study.

### Eligibility

Patients from around the world are considered for enrollment in the trial, and professional translators are available if needed. This indicates a broad scope of recruitment, offering the opportunity for participation to a diverse group of patients.

### Follow-Up Duration

Each participant in the trial is followed for safety and efficacy for 5 years post-dosing. For about the first 6 months following the gene therapy administration, participants are required to reside close to the clinical trial site for monitoring. After this period, the frequency of visits to the clinical trial site decreases, including both in-person and telephone assessments over the 5-year duration.

Regarding potential risks and benefits, participants in the trial are closely monitored for any side effects or adverse reactions related to the gene therapy. The primary goal of the trial is to assess the safety, tolerability, and efficacy of NGN-401 in treating Rett Syndrome. The therapy has been designed to avoid the toxicity associated with overexpression seen in conventional gene therapies, aiming to provide a controlled and consistent expression of the therapeutic gene.

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*

April 18, 2023

Dear Rett Syndrome Community,

Earlier this year Neurogene shared that the FDA cleared our Investigational New Drug (IND) application for NGN-401 gene therapy for the treatment of female children with Rett syndrome. NGN-401 is an investigational adeno-associated virus (AAV) gene therapy, using Neurogene's Expression Attenuation via Construct Tuning (EXACT) gene regulation technology. NGN-401 contains a full-length human *MECP2* gene which is designed to express therapeutic levels of the MECP2 protein while avoiding overexpression.

Since that announcement, we have received requests from the community, seeking additional information about the clinical trial. The purpose of this letter is to provide additional details. Following, are frequently asked questions we've received:

***What age range will be studied in this first clinical trial for NGN-401?***

- This clinical trial will study the investigational gene therapy, NGN-401, in females with a confirmed diagnosis of typical Rett syndrome, aged 4-10 years old.

***Will there be a clinical trial for a broader range of ages or for boys?***

- Findings from this clinical trial will inform decisions regarding additional clinical trials or expansion of enrollment criteria.

***How many participants will be included in the clinical trial, and will all participants receive NGN-401?***

- This initial phase of the clinical trial will enroll 5 participants.
- As the clinical trial progresses, we anticipate enrolling additional participants.
- All participants will receive the investigational gene therapy, NGN-401.

***Where will the clinical trial be conducted?***

- We do not yet have the final details to share about the clinical trial site(s), as it takes time to prepare and finalize logistics with the sites.
- The clinical trial will be conducted at hospitals (clinical trial sites) in the US, and will be led by a team of medical experts who have a deep knowledge of gene therapy and experience caring for individuals with Rett syndrome.

***Can families living outside of the United States enroll in the US clinical trial?***

- Not at this time. We are early in the process of working with regulators to explore the opportunity for additional clinical trial sites outside the US. We will provide further information once it becomes available.

***Will families be required to live near the clinical trial site?***

- Families enrolled in the clinical trial will be required to live near the trial site for at least the first 3 months after dosing.
- Living near the clinical trial site is important to monitor safety and will be less disruptive to the daily lives of families, given the multiple in-person follow-up visits required throughout the course of the trial.

***When will the clinical trial start enrolling participants?***

- We expect enrollment to begin in the Summer/Fall of 2023.

***Will clinical trial participants be allowed to be on trofinetide or in another clinical trial?***

- Currently, for this clinical trial, participants who are taking, or have ever taken, trofinetide or who are in another clinical trial will not be eligible for enrollment.
- For more information, please click HERE

***When will Neurogene provide additional details about the clinical trial?***

- Once the clinical trial is open for enrollment, we will send another communication to the community.
- Details will also be provided on www.clinicaltrials.gov, including the clinical trial site(s) and location(s).

***Can families contact someone now to express their interest in being in the clinical trial?***

- The clinical trial site details are not yet available, and there are no means to contact a site yet.
- The site(s) need to have the proper processes, training, resources and staffing in place before they can receive incoming interest from families.
- Neurogene, as the sponsor of the study, is unable to keep a list of interested families due to regulatory, legal, and compliance standards related to the conduct of a clinical trial.

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

Neurogene contact information is:

- By phone: +1-877-237-5020
- Patients and families can reach us at: patientinfo@neurogene.com
- Healthcare providers can reach us at: medicalinfo@neurogene.com
- Our website is: www.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

We truly appreciate your patience as we work to begin enrollment in the clinical trial as soon as possible. We are also appreciative of the questions and interest we have received since our announcement of the IND clearance for NGN-401. We are committed to providing you with information as it becomes available. We will share future updates about the clinical trial with the Rett syndrome community and patient advocacy organizations, on our website, and through our social media channels.

Sincerely,

*Kimberly Trant, RN, MBA*
*Executive Director, Patient Advocacy and Engagement*

January 23, 2023

Dear Rett Syndrome Community,

Today we are excited to share the news that the FDA has cleared Neurogene's Investigational New Drug (IND) application for NGN-401 for the treatment of children with Rett syndrome. NGN-401 is an investigational adeno-associated virus (AAV) gene therapy candidate to be administered to pediatric patients that uses Neurogene's Expression Attenuation via Construct Tuning (EXACT) gene regulation technology. The full press release issued today may be found at https://www.neurogene.com/news/

We realize you may have several questions upon hearing this news. The purpose of this letter is to help answer some of those questions.

First, Neurogene would like to express our gratitude to the entire Rett syndrome community for your recent participation in the survey shared by the Rett Syndrome Research Trust (RSRT) and the International Rett Syndrome Foundation (IRSF), as well as the many families we had the honor to speak with after completing the survey. Nearly 200 responses were received within the first few hours of the survey launching online! Your enthusiastic response is appreciated and was added to the input received from Rett syndrome clinical experts and the FDA, which helped to inform the clinical trial design, the IND submission, and our continued learning about how Rett syndrome impacts individuals and their families. We are also grateful to the Rett syndrome patient organizations in the US, the International Rett Syndrome Foundation and the Rett Syndrome Research Trust, for providing expertise and input which was critical to us reaching this important milestone.

***What does a cleared IND mean for a clinical trial?***

- Clearance of an IND by the FDA is a major milestone in advancing development of a new, investigational treatment
- It means that the US regulatory agency, the FDA, has provided approval for Neurogene to begin a clinical trial of the investigational gene therapy, NGN-401

***What age range will be studied in this first clinical trial for NGN-401?***

- This first clinical trial will study the investigational gene therapy, NGN-401, in female children with Rett syndrome
- Additional details will be available when enrollment begins

***Will there be a clinical trial for boys?***

- For this initial clinical trial, the FDA has approved initiation of a clinical trial in pediatric females with Rett syndrome
- Decisions regarding additional clinical trials will be shared with the Rett community when the information becomes available

***How many participants will be included in the clinical trial?***

- This initial clinical trial will enroll a limited number of participants; more details will be provided once enrollment begins. These details will be posted on clinicaltrials.gov once they become available.

***Where will the clinical trial be conducted?***

- The clinical trial will be conducted at a hospital (clinical trial site) in the US, and will be led by a team of medical experts who have a deep knowledge of gene therapy and experience caring for individuals with Rett syndrome; there will be more than one clinical trial site
- Neurogene will provide an update once clinical trial sites are ready to begin enrolling participants
- We are early in the process of working with regulators to explore the opportunity for additional clinical trial sites outside the U.S.; it is premature to provide further information at this time

***When will the clinical trial start enrolling participants?***

- The NGN-401 Phase 1/2 clinical trial to dose female pediatric patients with Rett syndrome, will be initiated in 2023
- Clinical trial sites have several processes to complete before they can enroll and dose the first participant
- Given the novel and complex nature of gene therapy, this may take several months; it is typical to take 6-12 months from FDA IND clearance until the first patient is dosed
- Therefore, we do not have exact timing to share now
- As these preparatory steps near completion, we will provide an update sharing additional information including when study enrollment will begin

***Can families contact someone now to express their interest in being in the clinical trial?***

- The clinical trial is not yet enrolling; once information about the sites becomes available, we will share contact information with the Rett syndrome patient advocacy organizations, as well as on our website and social media channels
- Neurogene, as the sponsor of the study, is unable to keep a list of interested families due to regulatory, legal, and compliance standards related to performing a clinical trial

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

Neurogene contact information is:

- By phone: +1-877-237-5020
- Patients and families can reach us at: patientinfo@neurogene.com
- Healthcare providers can reach us at: medicalinfo@neurogene.com
- Our website is: www.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 for the opportunity to advance our development program and begin our first clinical trial for Rett syndrome. We know that families are waiting for treatment options, so we will continue to work with a sense of urgency. We will share available updates on the clinical trial with the patient advocacy organizations, and through our social media channels. We remain committed to providing you with information as it becomes available. We appreciate the insights you have provided to help inform our work; it is an honor to work with this amazing community.

Sincerely,

*Kimberly Trant, RN, MBA*
*Executive Director, Patient Advocacy and Engagement*

June 12, 2023

Dear Rett Syndrome Community,

The purpose of this letter is to provide information about Neurogene's Rett syndrome clinical trial and to communicate the first clinical trial site location.

This first-in-human investigational gene therapy clinical trial is titled: ***A Phase 1/2, Open-Label Clinical Study to Evaluate Safety, Tolerability, and Efficacy of NGN-401 in Pediatric Subjects with Rett Syndrome***. This clinical trial will test NGN-401, an investigational adeno-associated virus (AAV) gene therapy that contains a full-length human *MECP2* gene and Neurogene's Expression Attenuation via Construct Tuning (EXACT) transgene regulation technology, in females with Rett syndrome. EXACT gene regulation is designed to express a controlled amount of MeCP2 protein.

**The clinical trial is enrolling participants at Texas Children's Hospital.**

**More details about the clinical trial and the site contact information are available at**:
https://clinicaltrials.gov/ct2/show/NCT05898620

- This link will be updated with clinical trial site information as additional site locations are opened in the future.

Since this is the first time the investigational gene therapy NGN-401 will be given to humans, the clinical trial will start with a small group of 5 participants. After safety assessments in this group are complete, we will make an informed decision about how to expand the clinical trial for additional participants to enroll.

## About the Phase 1/2 Investigational Gene Therapy Clinical Trial for Rett Syndrome

- **This is a prospective, open-label clinical trial**, which means all participants will receive the investigational gene therapy, NGN-401, and will be followed for 5 years, with an additional long-term follow-up for 10 years.
- The investigational gene therapy, NGN-401, will be given as a single **intracerebroventricular (ICV) injection into a ventricle of the brain.**
- This clinical trial will study the investigational gene therapy, NGN-401, in **5 females aged 4-10 years old**, with a diagnosis of typical Rett syndrome with a documented disease-causing mutation in the *MECP2* gene.
    - Participants are similar in age and stage of disease progression to better understand the safety and clinical effects of the investigational gene therapy.
- Individuals with **normal hand function will be excluded** (e.g., holding a pen/pencil effectively and/or drawing a shape).
- Individuals who are **participating in another clinical trial for an investigational medicine** will not be eligible for enrollment.
- **Each participant will be followed for safety and efficacy for 5 years after dosing.**
    - For approximately the first **3 months after the investigational gene therapy is given, families will be required to live (or temporarily relocate) within a 2-hour drive of the clinical trial site**.
    - Living near the clinical trial site is important to monitor safety and for multiple in-person follow-up visits required during the first three months.
    - After the initial safety monitoring period, there will be telephone and in-person visits with the clinical trial site in decreasing frequency over the rest of the 5-year period.
    - After the 5-year period, **it is expected that participants will enroll in a separate long-term observational study that will continue to collect information on safety and effects of the investigational treatment for 10 additional years.**

- **There is a comprehensive travel and expense policy in place to cover trial-related costs and expenses for participating families.**
    - Trial-related costs and expenses are paid by Neurogene; more details on the specific policy can be provided by the clinical trial site.
- **As with any clinical trial, participants may or may not benefit from this research.** There are potential risks, and there is no guarantee that being in this study will help the participant.

***Can families living outside of the United States enroll in the US clinical trial?***

- Not at this time. We are early in the process of working with regulators on the opportunity to add clinical trial sites outside the US. We will provide further information once it becomes available.

***Can families contact someone now to express their interest in being in the clinical trial?***

- **Interested families should contact a clinical trial site that is currently enrolling to express their interest.**

Additional information about the clinical trial and trial site(s) may be found at this link: https://clinicaltrials.gov/ct2/show/NCT05898620

Previous letters to the Rett syndrome community, including frequently asked questions from the community, can be viewed at: https://www.neurogene.com/patients-and-families/

Sincerely,

*Kimberly Trant, RN, MBA*
Executive Director, Patient Advocacy and Engagement
www.neurogene.com
**Follow us on: LinkedIn | Twitter | Facebook**

# Neurogene Announces Closing of Merger with Neoleukin Therapeutics and Concurrent Private Placement of $95 Million

December 19, 2023

*Neurogene focused on advancing Phase 1/2 trial for NGN-401, a differentiated clinical stage gene therapy to treat Rett syndrome using its EXACT technology; interim clinical data expected in 4Q24*

*Two patients successfully dosed with NGN-401, which has been well tolerated to date with no treatment-emergent or procedure-related serious adverse events, or transgene-related overexpression toxicity*

*Post transaction cash, cash equivalents, and investments of approximately $200 million expected to fund advancement of Neurogene's EXACT gene therapy portfolio into 2H26*

*Shares to trade on NASDAQ under the new ticker "NGNE"*

NEW YORK--(BUSINESS WIRE)--Dec. 19, 2023-- Neurogene Inc. (NASDAQ: NGNE) ("Neurogene"), a clinical-stage company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, today announced the closing of its merger with Neoleukin Therapeutics, Inc. ("Neoleukin"). Neurogene shares are expected to begin trading on the NASDAQ Global Market under the ticker "NGNE" beginning today at the market open.

Concurrent with the closing of the merger, Neurogene closed an oversubscribed $95 million private financing, led by new and existing healthcare-dedicated specialist and mutual fund institutional investors, including participation from Great Point Partners, EcoR1 Capital, Redmile Group, Samsara BioCapital, Janus Henderson Investors, funds and accounts managed by Blackrock, Casdin Capital, Avidity Partners, Arrowmark Partners, Cormorant Asset Management, Alexandria Venture Investments, and a healthcare investment fund. Neurogene's cash, cash equivalents, and investments of approximately $200 million, before payment of final transaction-related expenses, are expected to fund operations and multiple potentially value-creating milestones into the second half of 2026.

"This transformative transaction provides us with a strong cash position allowing us to demonstrate the best-in-class potential of our EXACT transgene regulation technology in treating Rett syndrome, a debilitating and complex neurological disease that cannot be treated with conventional gene therapy," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We look forward to expanding our ongoing Phase 1/2 clinical trial in 2024 for pediatric patients with Rett syndrome beyond the first cohort of five patients, and presenting interim clinical data from this study in the fourth quarter of 2024, with additional data from an expanded number of patients expected in the second half of 2025."

NGN-401 is an investigational adeno-associated virus (AAV9) gene therapy candidate for Rett syndrome purposefully designed and administered to maximize therapeutic activity while averting transgene overexpression toxicities. NGN-401 delivers the full-length human methyl cytosine binding protein 2 (*MECP2*) gene, providing an optimal gene replacement approach. NGN-401 leverages Neurogene's novel and proprietary EXACT transgene regulation technology, which provides a highly controlled and consistent MeCP2 expression on a cell by cell basis, and avoids overexpression-related toxicities associated with conventional gene therapy.

Neurogene recently announced the dosing of the first two patients with NGN-401 in the third and fourth quarters of 2023. Data from the ongoing Phase 1/2 clinical trial demonstrate that NGN-401 has been well tolerated, with no treatment-emergent serious adverse events or procedure-related events, and no signs of treatment-related overexpression toxicity. NGN-401 has been granted Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track Designation by the U.S. Food and Drug Administration (FDA).

Neurogene is also developing NGN-101 for the treatment of CLN5 Batten disease, with interim clinical data for NGN-101 expected in the second half of 2024, and is advancing multiple discovery-stage candidates leveraging its EXACT transgene regulation technology. Neurogene expects to initiate a clinical study of one product candidate from its discovery-stage portfolio in 2025.

**Transaction Details**

To ensure the combined company's compliance with the minimum bid price requirement of $4.00 per share for initial listing on The Nasdaq Global Market, Neoleukin implemented a reverse split of its common stock at a ratio of 1-for-4 shares. In the reverse stock split, every four shares of Neoleukin common stock outstanding were combined and reclassified into one share of Neoleukin common stock. Immediately thereafter, and pursuant to the terms of the previously announced merger agreement, Neurogene became a wholly owned subsidiary of Neoleukin upon completion of the merger, and each outstanding share of Neurogene common stock was converted into 0.0756 shares of Neoleukin common stock. Following the closing of the merger, there are approximately 16,887,060 shares of the combined company's common stock outstanding (assuming the exercise in full of all pre-funded warrants), with prior Neurogene stockholders, including investors in the private placement, owning approximately 84% and prior Neoleukin stockholders owning approximately 16% of the combined company's outstanding securities. The combined company will be led by Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene, and other members of the Neurogene management team.

TD Cowen served as exclusive financial advisor to Neurogene. TD Cowen and Stifel served as placement agents for Neurogene's concurrent private financing. Gibson Dunn & Crutcher LLP served as legal counsel to Neurogene and Cooley LLP served as legal counsel to the placement agents. Leerink Partners served as the exclusive financial advisor to Neoleukin. Fenwick & West LLP served as legal counsel to Neoleukin.

**About Neurogene**

The mission of Neurogene is to treat devastating neurological diseases to improve the lives of patients and families impacted by these rare diseases. Neurogene is developing novel approaches and treatments to address the limitations of conventional gene therapy in central nervous system disorders. This includes selecting a delivery approach to maximize distribution to target tissues and by designing products to maximize potency and purity for an optimized efficacy and safety profile. The Company's novel and proprietary EXACT transgene regulation platform technology allows for

the delivery of therapeutic levels while limiting transgene toxicity associated with conventional gene therapy. Neurogene has constructed a state-of-the-art gene therapy manufacturing facility in Houston, Texas. CGMP production of NGN-401 was conducted in this facility and will support pivotal clinical development activities. For more information, visit www.neurogene.com.

**Cautionary Note Regarding Forward-Looking Statements**

This communication contains forward-looking statements (including within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended (Securities Act). These statements may discuss goals, intentions and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current expectations and beliefs of the management of Neurogene, as well as assumptions made by, and information currently available to, management of Neurogene. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Statements that are not historical facts are forward-looking statements. Forward-looking statements in this communication include, but are not limited to, statements regarding the expected enrollment of and timing of data from Neurogene's Phase 1/2 clinical trial; statements regarding the potential of, and expectations regarding, Neurogene's programs, including NGN-101, NGN-401 and its research stage opportunities; the expected dosing of additional patients in Neurogene's Phase 1/2 clinical trial; statements by Neurogene's Founder and Chief Executive Officer; statements regarding the sufficiency of Neurogene's capital resources and cash runway. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: Neurogene's limited operating history; the significant net losses incurred since inception of Neurogene; the ability to raise additional capital to finance operations; the ability to advance product candidates through non-clinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Neurogene's product candidates; the outcome of non-clinical testing and early clinical trials for Neurogene's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; Neurogene's limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the market and potential for Neurogene's current product candidates; the substantial competition Neurogene faces in discovering, developing, or commercializing products; expectations regarding the potential tolerability, safety or efficacy for Neurogene's current product candidates; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Neurogene to protect its intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; and legislative, regulatory, political and economic developments and general market conditions. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in Neoleukin's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the Securities and Exchange Commission (SEC), the registration statement on Form S-4 filed with the SEC by Neoleukin and other documents to be filed by Neurogene from time to time with the SEC, discussions of potential risks, uncertainties and other important factors in Neurogene's subsequent filings with the SEC, as well as risk factors associated with companies, such as Neurogene, that operate in the biopharma industry. These forward-looking statements involve a number of risks, uncertainties (some of which are beyond Neurogene control) or other assumptions that may cause actual results or performance to be materially different from those expressed or implied by these forward-looking statements. Nothing in this communication should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that the contemplated results of any such forward-looking statements will be achieved. Forward-looking statements in this communication speak only as of the day they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by applicable law, Neurogene undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

This communication contains hyperlinks to information that is not deemed to be incorporated by reference into this communication.

View source version on businesswire.com: https://www.businesswire.com/news/home/20231219056613/en/

**Neurogene Contacts:**

Investor Relations:
Melissa Forst
Argot Partners
Neurogene@argotpartners.com

Media:
David Rosen
Argot Partners
david.rosen@argotpartners.com

Source: Neurogene Inc.

# Neurogene Doses First Patients in Phase 1/2 Trial of NGN-401 for the Treatment of Female Pediatric Patients with Rett Syndrome

November 30, 2023

*Two pediatric patients with Rett syndrome dosed in the United States with NGN-401, Neurogene's lead gene therapy product candidate leveraging its proprietary EXACT gene regulation technology*

*NGN-401 has been well-tolerated to date with no treatment-emergent or procedure-related serious adverse events (SAEs) or transgene-related overexpression toxicity*

*Third patient dosing anticipated in 1Q24, and interim efficacy data from multiple patients in the first cohort remains on track for 4Q24*

*NGN-401 design and delivery approach optimized to maximize efficacy and safety profile*

**New York, NY – November 30, 2023 –** Neurogene Inc., a clinical-stage company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, announced today the dosing of the first two female pediatric patients with Rett syndrome in its ongoing Phase 1/2 trial of NGN-401. Rett syndrome is a debilitating, X-linked, neurodevelopmental disorder with significant unmet medical need. To date, NGN-401 has been well-tolerated with no treatment-emergent or procedure-related SAEs, and no signs of transgene-related overexpression toxicity.

NGN-401 is an investigational adeno-associated virus (AAV) gene therapy candidate for Rett syndrome purposefully designed and administered to maximize the therapeutic activity while averting transgene overexpression toxicities. NGN-401 delivers the full-length human methyl cytosine binding protein 2 (*MECP2*) gene, providing an optimal gene replacement approach. Moreover, NGN-401 leverages Neurogene's novel and proprietary Expression Attenuation via Construct Tuning (EXACT) gene regulation technology, which provides highly controlled and consistent MeCP2 expression on a cell-by-cell basis, thus avoiding the overexpression related toxicities associated with conventional gene therapy. In non-clinical studies with NGN-401 at clinically relevant doses, cardinal features of Rett syndrome were ameliorated, and no overexpression toxicity was observed. These data were part of the robust non-clinical package that supported the U.S. Food and Drug Administration's (FDA) decision to allow Neurogene to proceed directly into a pediatric population for its first-in-human study. NGN-401 has also been granted Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track Designation by the FDA.

"While gene therapy has proven to be a powerful tool in the treatment armamentarium for a number of devastating genetic conditions, the highly variable transgene expression associated with conventional gene therapies has limited its application in many complex neurological disorders, especially in Rett syndrome, in which *MECP2* transgene overexpression is toxic," said Bernhard Suter, M.D., Principal Investigator of the Phase 1/2 clinical trial, and Associate Professor of Pediatrics and Neurology at Baylor College of Medicine and Texas Children's Hospital. "NGN-401 has been well tolerated to date in the first two patients dosed, consistent with the wide safety margins established in non-clinical studies conducted in disease models and in normal non-human primates."

**Ongoing Phase 1/2 Trial Update**

The first-in-human, open-label, single-arm, multi-center Phase 1/2 clinical trial (NCT05898620) is evaluating NGN-401 at a dose of $1\times10^{15}$ total vector genomes to assess the safety and tolerability of NGN-401 in female pediatric patients ages 4-10 with Rett syndrome. NGN-401 is administered as a one-time treatment using intracerebroventricular (ICV) administration, which has been shown to maximize the delivery of the therapeutic *MECP2* gene to key areas of the brain underlying Rett syndrome pathobiology. Clinical-grade NGN-401 for this trial was manufactured at Neurogene's Good Manufacturing Practices (GMP) facility.

The first two patients were dosed sequentially in the third and fourth quarter of 2023 at Texas Children's Hospital, an internationally recognized pediatric research center affiliated with Baylor College of Medicine, and the first clinical trial site to be opened in the U.S. for this study. Dr. Daniel Curry, M.D., director, Functional Neurosurgery and Epilepsy Surgery at Texas Children's Hospital and professor, Neurosurgery and Surgery at Baylor College of Medicine, performed the procedure to administer the gene therapy.

NGN-401 has been well-tolerated to date, with no treatment-emergent or procedure-related SAEs, and no observations of transgene-related overexpression. Pending successful completion of the trial's upcoming pre-planned independent Data and Safety Monitoring Board review, Neurogene expects to dose a third patient in the first quarter of 2024. The first cohort is expected to enroll a total of five female pediatric patients, with a planned expansion pending additional data and subject to review by health authorities.

"NGN-401 was purposefully designed to deliver a therapeutic benefit with the full length MECP2 gene, avoid toxicity associated with overexpression, and leverage the ICV route of delivery to maximize the biodistribution of the transgene to key areas of the brain underlying Rett syndrome. Based on published peer-reviewed non-clinical research, we know that Rett syndrome is caused by loss of function of *MECP2* in the brain and spinal cord, and therefore we believe delivering robust transgene expression in these areas is essential for enabling a clinically meaningful benefit," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "We are encouraged by the tolerability profile observed in our first two pediatric patients, and look forward to collecting sufficient follow up data on a larger number of patients to inform the therapeutic potential of NGN-401, which we believe could serve as a best-in-class therapy. We remain on track to report preliminary clinical data from the first cohort of patients in this trial in the fourth quarter of 2024, with additional data from an expanded number of patients expected in the second half of 2025."

Dr. McMinn added, "On behalf of the entire Neurogene team, we extend our gratitude to all those making this study possible, with special appreciation for the patients and their families. Your resilience, courage, and support not only contribute to the progress of this research, but also inspire hope within the entire Rett syndrome community. Together, we have the potential to make a meaningful impact on improving the many lives affected by this devastating disease."

**About EXACT**

Neurogene's novel and proprietary EXACT gene regulation platform technology is a self-contained transgene regulation platform that can be tuned to deliver a desired level of transgene expression within a narrow and therapeutically relevant range, with the goal of avoiding transgene-related toxicities associated with conventional gene therapy. EXACT is compatible with viral and non-viral delivery platforms.

**About NGN-401**

NGN-401 is an investigational AAV9 gene therapy being developed as a one-time treatment for Rett syndrome. It is the first clinical candidate to deliver the full-length human *MECP2* gene under the control of Neurogene's EXACT technology. The EXACT technology utilized in NGN-401 is an important advancement in gene therapy for Rett syndrome, specifically because the disorder requires a treatment approach that enables targeted levels of *MECP2* transgene expression without causing overexpression-related toxic effects associated with conventional gene therapy. The robust non-clinical data package for NGN-401 provides evidence of a potentially compelling efficacy and safety profile in Rett syndrome.

**About Rett Syndrome**

Rett syndrome is a rare genetic disorder that occurs almost exclusively in females, and leads to severe impairments that affect nearly every aspect of their lives. This includes their ability to speak, walk, eat, and breathe. The impact on patients and families is profound. Most females are nonverbal, lack motor skills, and require 24-hour care for all activities of daily living. Many females with Rett syndrome appear to understand the world around them, leaving many caregivers feeling that their daughters are intellectually and emotionally intact.

Rett syndrome is an X-linked, progressive, neurodevelopmental disorder. It has an estimated worldwide incidence of 1 out of every 10,000-15,000 live female births.

The incidence in males is currently unknown. Advances in genetic testing and phenotypic identification have revealed that *MECP2* mutations in males are responsible for a wide spectrum of neurological disorders, including Rett syndrome.

Rett syndrome is caused by mutations in the *MECP2* gene that lead to deficiency of the methyl cytosine binding protein 2, an important protein responsible for normal function in the brain and other parts of the nervous system.

Females with Rett syndrome typically have normal development until 6-18 months of age, followed by a progressive deterioration of acquired skills such as gross and fine motor skills, purposeful hand function and communication. They subsequently develop stereotypic hand movements such as hand-wringing.

Over time females may develop muscle contractures, rigidity, and debilitating scoliosis, along with periods of recurrent seizures, and burdensome gastrointestinal and breathing abnormalities.

Although there are treatments available for Rett syndrome, there is no treatment option that addresses the root cause of disease and a significant unmet need still exists for new treatment options.

**About Neurogene**

The mission of Neurogene is to treat devastating neurological diseases to improve the lives of patients and families impacted by these rare diseases. Neurogene is developing novel approaches and treatments to address the limitations of conventional gene therapy in central nervous system disorders. This includes selecting a delivery approach to maximize distribution to target tissues and designing products to maximize potency and purity for an optimized efficacy and safety profile. The Company's novel and proprietary EXACT gene regulation platform technology allows for the delivery of therapeutic levels while limiting transgene toxicity associated with conventional gene therapy. Neurogene has constructed a state-of-the-art gene therapy manufacturing facility in Houston, Texas. GMP production of NGN-401 was conducted in this facility and will support pivotal clinical development activities. For more information, visit www.neurogene.com.

**Neurogene Contacts:**

Investor Relations:
Melissa Forst
Argot Partners
Neurogene@argotpartners.com

Media:
David Rosen
Argot Partners
david.rosen@argotpartners.com

**Cautionary Note Regarding Forward-Looking Statements**

This communication contains forward-looking statements (including within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended (Securities Act). These statements may discuss goals, intentions and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current expectations and beliefs of the management of Neurogene, as well as assumptions made by, and information currently available to, management of Neurogene. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Statements that are not historical facts are forward-looking statements. Forward-looking statements in this communication include, but are not limited to, statements regarding the expected enrollment of and timing of data from Neurogene's Phase 1/2 clinical trial; statements regarding the potential of, and expectations regarding, Neurogene's programs, including NGN-101, NGN-401 and its research stage opportunities; the expected dosing of additional patients in Neurogene's Phase 1/2 clinical trial; statements by Neurogene's Founder and Chief Executive Officer. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various

factors, including, without limitation: Neurogene's limited operating history; the significant net losses incurred since inception of Neurogene; the ability to raise additional capital to finance operations; the ability to advance product candidates through non-clinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Neurogene's product candidates; the outcome of non-clinical testing and early clinical trials for Neurogene's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; Neurogene's limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the market and potential for Neurogene's current product candidates; the substantial competition Neurogene faces in discovering, developing, or commercializing products; expectations regarding the potential tolerability, safety or efficacy for Neurogene's current product candidates; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Neurogene to protect its intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; Neurogene's ability to consummate the proposed merger transactions with Neoleukin Therapeutics, Inc. (Neoleukin); the risk that the conditions to the closing of the proposed transactions are not satisfied, including the failure to obtain stockholder approval for the proposed transactions from Neoleukin's stockholders or to complete the transactions in a timely manner or at all; uncertainties as to the timing of the consummation of the proposed transactions; risks related to Neoleukin's continued listing on the Nasdaq Capital Market until closing of the proposed transactions; risks related to Neoleukin's and Neurogene's ability to correctly estimate their respective operating expenses and expenses associated with the proposed transactions, as well as uncertainties regarding the impact any delay in the closing would have on the anticipated cash resources of the combined company upon closing and other events and unanticipated spending and costs that could reduce the combined company's cash resources; the occurrence of any event, change or other circumstance or condition that could give rise to the termination of the merger agreement or Neurogene's financing transaction; competitive responses to the proposed transactions; unexpected costs, charges or expenses resulting from the proposed transactions; the outcome of any legal proceedings that may be instituted against Neoleukin, Neurogene or any of their respective directors or officers related to the merger, the financing transaction, or the proposed transactions contemplated thereby; the expected trading of the combined company's stock on Nasdaq Capital Market under the ticker symbol "NGNE" and the combined company's ability to remain listed following the proposed transactions; and legislative, regulatory, political and economic developments and general market conditions. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in Neoleukin's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the Securities and Exchange Commission (SEC), the registration statement on Form S-4 filed with the SEC by Neoleukin, as well as risk factors associated with companies, such as Neurogene, that operate in the biopharma industry. There can be no assurance that the conditions of the proposed transactions will be satisfied or that future developments affecting Neurogene, Neoleukin or the proposed transactions will be those that have been anticipated. These forward-looking statements involve a number of risks, uncertainties (some of which are beyond Neurogene and Neoleukin's control) or other assumptions that may cause actual results or performance to be materially different from those expressed or implied by these forward-looking statements. Nothing in this communication should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that the contemplated results of any such forward-looking statements will be achieved. Forward-looking statements in this communication speak only as of the day they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by applicable law, Neurogene undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

This communication contains hyperlinks to information that is not deemed to be incorporated by reference into this communication.

**Important Additional Information About the Proposed Transactions Has Been Filed with the SEC**

This communication is not a substitute for the registration statement or for any other document that Neoleukin has filed with the SEC in connection with the proposed transactions. In connection with the proposed transactions, Neoleukin has filed a registration statement on Form S-4 that contains a proxy statement/prospectus of Neoleukin. NEOLEUKIN URGES INVESTORS AND STOCKHOLDERS TO READ THE REGISTRATION STATEMENT, PROXY STATEMENT/PROSPECTUS AND ANY OTHER RELEVANT DOCUMENTS THAT MAY BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THESE DOCUMENTS, CAREFULLY AND IN THEIR ENTIRETY BECAUSE THEY CONTAIN IMPORTANT INFORMATION ABOUT NEOLEUKIN, NEUROGENE, THE PROPOSED TRANSACTIONS AND RELATED MATTERS. Investors and stockholders can obtain free copies of the proxy statement/prospectus and other documents filed by Neoleukin with the SEC through the website maintained by the SEC at www.sec.gov. In addition, investors and stockholders should note that Neoleukin communicates with investors and the public using its website (www.neoleukin.com), the investor relations website (https://investors.neoleukin.com/) where anyone can obtain free copies of the proxy statement/prospectus and other documents filed by Neoleukin with the SEC and stockholders are urged to read the proxy statement/prospectus and the other relevant materials before making any voting or investment decision with respect to the proposed transactions.

**Participants in the Solicitation**

Neoleukin, Neurogene and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about Neoleukin's directors and executive officers who may, under the rules of the SEC, be deemed participants in the solicitation of the stockholders of Neoleukin in connection with the proposed transaction, including a description of their direct or indirect interests, by security holdings or otherwise, is set forth in the proxy statement/prospectus included in the registration statement on Form S-4 initially filed by Neoleukin with the SEC on August 21, 2023, as subsequently amended on September 28, 2023, October 18, 2023 and November 8, 2023.

# Neurogene and Neoleukin Announce Definitive Merger Agreement

July 18, 2023

*Proposed merger to create Nasdaq-listed biotech company focused on advancing Neurogene's differentiated portfolio of genetic medicines for complex neurological diseases*

*Combined company is expected to have a cash balance of approximately $200 million at close, including approximately $95 million from concurrent private financing by Neurogene's new and existing investors*

*Cash expected to fund combined company into 2H:26 and through multiple catalysts, including preliminary data in 4Q:24 and additional data in 2H:25 from a Phase 1/2 clinical trial in Rett syndrome*

*Companies to host conference call today at 8:30 am ET*

**New York, NY and Seattle, WA – July 18, 2023 –** Neurogene Inc., a clinical-stage company founded to bring life-changing genetic medicines to patients and families affected by rare neurological diseases, and Neoleukin Therapeutics, Inc. (NASDAQ:NLTX) today announced that they have entered into a definitive merger agreement to combine the companies in an all-stock transaction. The combined company will focus on advancing Neurogene's pipeline of differentiated genetic medicines, including NGN-401, a clinical-stage product for Rett syndrome, which uses novel gene regulation technology for a potential best-in-class profile. Upon completion of the merger, which is subject to approval by Neurogene and Neoleukin stockholders, the combined company is expected to operate under the name Neurogene Inc. and trade on the Nasdaq Capital Market under the ticker symbol "NGNE".

In connection with the merger, Neurogene announced an oversubscribed $95 million private financing led by new and existing healthcare-dedicated specialist and mutual fund institutional investors, including participation from Great Point Partners, EcoR1 Capital, Redmile Group, Samsara BioCapital, Janus Henderson Investors, funds and accounts managed by Blackrock, Casdin Capital, Avidity Partners, Arrowmark Partners, Cormorant Asset Management, Alexandria Venture Investments, and a healthcare investment fund.

With the cash from both companies at closing and the proceeds of the concurrent private financing, the combined company is expected to have approximately $200 million of cash or cash equivalents immediately following the closing. The cash resources are intended to be used to advance Neurogene's pipeline through multiple clinical milestones and are expected to fund operations into the second half of 2026. The merger and concurrent private financing are expected to close in the fourth quarter of 2023, subject to stockholder approval of both companies, the effectiveness of a registration statement to be filed with the U.S. Securities and Exchange Commission to register the securities to be issued in connection with the merger and concurrent financing, and the satisfaction of customary closing conditions.

"We are excited to announce our planned merger with Neoleukin, which we believe is a transformative step forward in our mission to bring life-changing genetic medicines to the patients and families impacted by devastating neurological diseases," said Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene. "This transaction is expected to bolster our ability to progress our differentiated pipeline, including our clinical-stage program in Rett syndrome which contains our novel, proprietary EXACT technology. We believe EXACT represents a meaningful technological advance for the gene therapy field, allowing us to develop therapeutic product candidates for complex diseases with attractive market opportunities not addressable with conventional gene therapy. This capital will also support our internal manufacturing capabilities, which we expect will continue to provide significant financial and strategic flexibility. With cash on hand at the close of this transaction expected to fund operations into the second half of 2026, we believe we are well positioned to successfully execute beyond multiple anticipated clinical inflection points for both Rett syndrome and Batten disease, and advance our discovery stage pipeline."

"This merger with Neurogene reflects the continued commitment of our management team and Board of Directors to deliver value to stockholders and, importantly, meaningfully improve patients' lives," said Donna Cochener, Interim Chief Executive Officer and General Counsel of Neoleukin. "Neurogene has an innovative genetic medicines portfolio, in-house product design and manufacturing capabilities, an impressive management team, and will be well positioned to deliver multiple data readouts in the next 18 to 24 months. We are grateful to our current and former employees who contributed to Neoleukin's efforts and look forward to the combined company's continued progress and success."

**About Neurogene's Portfolio and EXACT Gene Regulation Platform**

Neurogene's internally manufactured portfolio of purposefully designed therapies aims to address several key limitations of conventional gene therapies, including variable gene expression, safety limitations, and inefficient gene delivery.

The company's novel and proprietary Expression Attenuation via Construct Tuning (EXACT) gene regulation platform technology is a self-contained transgene regulation platform that can be tuned to deliver a desired level of transgene expression within a narrow range, potentially avoiding transgene related toxicities associated with conventional gene therapy. EXACT is compatible with viral and non-viral delivery platforms.

Neurogene's clinical-stage portfolio includes:

**NGN-401:** NGN-401 is an investigational AAV9 gene therapy being developed as a one-time treatment for Rett syndrome. It is the first candidate to deliver the full-length human MECP2 gene under the control of Neurogene's EXACT technology. Embedding EXACT technology into NGN-401 is an important advancement in gene therapy for Rett syndrome, specifically because the disorder requires a treatment approach that enables targeted levels of MECP2 transgene expression without causing toxic effects associated with conventional gene therapy. Rett syndrome is a debilitating, X-linked, neurodevelopmental disorder with significant unmet medical need, and one of the most common genetic causes of developmental and intellectual impairment in females.

The robust preclinical data package for NGN-401 provides evidence of a potentially compelling efficacy and safety profile in Rett syndrome. The company's Investigational New Drug (IND) application was cleared by the U.S. Food and Drug Administration in January 2023. In the U.S., NGN-401

has received Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track designation. Neurogene plans to commence dosing in a Phase 1/2 trial (NCT05898620) designed to assess the safety, tolerability, and efficacy of a single dose of NGN-401 in female pediatric patients with Rett syndrome in the second half of 2023, with preliminary data expected in the fourth quarter of 2024 from the first cohort of patients, and additional expected data in the second half of 2025 from an expanded set of patients.

**NGN-101:** NGN-101 is being developed as a one-time treatment for both ocular and neurological manifestations of CLN5 Batten disease using AAV9 to deliver the gene encoding CLN5, which is deficient in children with the disease. Batten disease is a family of rare neurodegenerative diseases caused by pathogenic changes in one of a series of genes that results in the accumulation of toxic deposits across multiple organ systems. CLN5 Batten disease is a rare, pediatric-onset and rapidly progressive condition caused by a pathogenic mutation in the CLN5 gene, leading to loss of function. It is characterized by loss of vision, seizures, and progressive decline in intellectual and motor capabilities beginning in childhood leading to substantial impairments and early mortality.

In preclinical studies, NGN-101 has demonstrated the potential to slow or halt the key features of disease progression, including associated vision and motor declines. NGN-101 has received Orphan Drug Designation by U.S. and European regulatory agencies and is currently being evaluated in a Phase 1/2 clinical trial in children with CLN5 Batten disease (NCT05228145). Preliminary data is expected in the second half of 2024.

In addition to these two clinical-stage programs, Neurogene is also advancing a discovery-stage candidate that will expand its pipeline into an additional area of high unmet need. Neurogene expects to initiate a clinical study of this candidate in 2025.

**About the Proposed Merger**

Under the terms of the merger agreement, Neoleukin will issue to pre-merger Neurogene stockholders shares of Neoleukin common stock as merger consideration in exchange for the cancellation of shares of capital stock of Neurogene, and Neurogene will become a wholly owned subsidiary of Neoleukin. Pre-merger Neoleukin stockholders are expected to own approximately 16% of the combined company and pre-merger Neurogene stockholders (including those purchasing Neurogene shares in the concurrent private financing discussed above) are expected to own approximately 84% of the combined company. The percentage of the combined company that pre-merger Neurogene stockholders and pre-merger Neoleukin stockholders will own as of the close of the proposed transaction is subject to certain adjustments as described in the merger agreement, including the amount of Neoleukin's net cash at closing. In connection with the closing of the proposed transactions, Neoleukin stockholders will also be issued contingent value rights representing the right to receive certain payments from proceeds received by the combined company, if any, related to Neoleukin's pre-transaction legacy assets or from savings realized by the combined company, if any, related to the reduction of Neoleukin's legacy lease obligations.

Upon closing of the proposed transaction, Neoleukin Therapeutics, Inc., will be renamed Neurogene Inc. The combined company will be led by Rachel McMinn, Ph.D., Founder and Chief Executive Officer of Neurogene, and other members of the Neurogene management team. The combined company's Board of Directors will be comprised of five board members selected by Neurogene and two members selected by Neoleukin. The transaction has been unanimously approved by the Board of Directors of each company and is expected to close in the fourth quarter of 2023, subject to customary closing conditions, including the approval of the transaction by the stockholders of each company.

TD Cowen is serving as exclusive financial advisor to Neurogene. TD Cowen and Stifel are serving as placement agents on Neurogene's planned concurrent private financing. Gibson Dunn & Crutcher LLP is serving as legal counsel to Neurogene and Cooley LLP is serving as legal counsel to the placement agents. Leerink Partners is serving as the exclusive financial advisor to Neoleukin. Fenwick & West LLP is serving as legal counsel to Neoleukin.

**Conference Call Information**

Neurogene and Neoleukin will host a conference call today, July 18, 2023, at 8:30 am E.T. to discuss the proposed merger. The live webcast can be accessed by visiting https://edge.media-server.com/mmc/p/q3vx354g. To access the event via phone, please register to receive a unique dial-in and PIN number using the following link: https://register.vevent.com/register/BI3014e8ea8bec4d9cbdf9a68a0b5c78ec

A replay of the webcast will be available for a limited time following the event on the Events & Presentations section of Neoleukin's website at https://investor.neoleukin.com/events and on the News section of Neurogene's website at https://www.neurogene.com/news/.

**About Neurogene**

The mission of Neurogene is to turn devastating neurological diseases into treatable conditions to improve the lives of patients and families impacted by these rare diseases. Neurogene is developing novel approaches and treatments to address the limitations of conventional gene therapy in central nervous system disorders. This includes selecting a delivery approach to maximize distribution to target tissues and by designing products to maximize potency and purity for an optimized efficacy and safety profile. The company's novel and proprietary EXACT gene regulation platform technology allows for the delivery of therapeutic levels while limiting transgene toxicity associated with conventional gene therapy. For more information, visit www.neurogene.com.

**About Neoleukin**

Neoleukin is a biopharmaceutical company creating next generation immunotherapies for cancer, inflammation and autoimmunity using de novo protein design technology. Neoleukin uses sophisticated computational methods to design proteins that demonstrate specific pharmaceutical properties that provide potentially superior therapeutic benefit over native proteins. For more information, please visit the Neoleukin website: www.neoleukin.com.

**Cautionary Note Regarding Forward-Looking Statements**

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stage opportunities; statements by Neoleukin's Interim Chief Executive Officer and General Counsel; and statements by Neurogene's Founder and Chief Executive Officer. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: the limited operating history of each company; the significant net losses incurred since inception of each company; the ability to raise additional capital to finance operations; the ability to advance product candidates through preclinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Neurogene's product candidates; the outcome of preclinical testing and early clinical trials for Neurogene's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; Neurogene's limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Neurogene's current product candidates; expectations regarding the market and potential for Neurogene's current product candidates; the substantial competition Neurogene faces in discovering, developing, or commercializing products; the negative impacts of the COVID-19 pandemic on operations, including ongoing and planned clinical trials and ongoing and planned preclinical studies; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Neoleukin or Neurogene to protect their respective intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; the risk that the conditions to the closing of the proposed transactions are not satisfied, including the failure to obtain stockholder approval for the proposed transactions from both Neoleukin and Neurogene's stockholders or to complete the transactions in a timely manner or at all; uncertainties as to the timing of the consummation of the proposed transactions and the ability of each of the parties to consummate the proposed transactions; risks related to Neoleukin's continued listing on the Nasdaq Capital Market until closing of the proposed transactions; risks related to Neoleukin's and Neurogene's ability to correctly estimate their respective operating expenses and expenses associated with the proposed transactions, as well as uncertainties regarding the impact any delay in the closing would have on the anticipated cash resources of the combined company upon closing and other events and unanticipated spending and costs that could reduce the combined company's cash resources; the occurrence of any event, change or other circumstance or condition that could give rise to the termination of the merger agreement or the financing transaction; competitive responses to the proposed transactions; unexpected costs, charges or expenses resulting from the proposed transactions; the outcome of any legal proceedings that may be instituted against Neoleukin, Neurogene or any of their respective directors or officers related to the merger, the financing transaction, or the proposed transactions contemplated thereby; potential adverse reactions of changes to business relationships resulting from the announcement or completion of the proposed transactions; the effect of the announcement or pendency of the transactions on Neoleukin's or Neurogene's business relationships, operating results and business generally; the expected trading of the combined company's stock on Nasdaq Capital Market under the ticker symbol "NGNE" and the combined company's ability to remain listed following the proposed transactions; and legislative, regulatory, political and economic developments and general market conditions. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors included in Neoleukin's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the SEC, the registration statement on Form S-4 to be filed with the SEC by Neoleukin, as well as risk factors associated with companies, such as Neurogene, that operate in the biopharma industry. There can be no assurance that the conditions of the proposed transactions will be satisfied or that future developments affecting Neurogene, Neoleukin or the proposed transactions will be those that have been anticipated. These forward-looking statements involve a number of risks, uncertainties (some of which are beyond Neurogene and Neoleukin's control) or other assumptions that may cause actual results or performance to be materially different from those expressed or implied by these forward-looking statements. Nothing in this press release should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that the contemplated results of any such forward-looking statements will be achieved. Forward-looking statements in this press release speak only as of the day they are made and are qualified in their entirety by reference to the cautionary statements herein. Except as required by applicable law, Neoleukin and Neurogene undertake no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference into this press release.

**No Offer or Solicitation**

This press release and the information contained herein is not intended to and does not constitute (i) a solicitation of a proxy, consent or approval with respect to any securities or in respect of the proposed transactions or (ii) an offer to sell or the solicitation of an offer to subscribe for or buy or an invitation to purchase or subscribe for any securities pursuant to the proposed transactions or otherwise, nor shall there be any sale, issuance or transfer of securities in any jurisdiction in contravention of applicable law. No offer of securities shall be made except by means of a prospectus meeting the requirements of the Securities Act of 1933, as amended, or an exemption therefrom.

Subject to certain exceptions to be approved by the relevant regulators or certain facts to be ascertained, the public offer will not be made directly or indirectly, in or into any jurisdiction where to do so would constitute a violation of the laws of such jurisdiction, or by use of the mails or by any means or instrumentality (including without limitation, facsimile transmission, telephone and the internet) of interstate or foreign commerce, or any facility of a national securities exchange, of any such jurisdiction.

NEITHER THE SEC NOR ANY STATE SECURITIES COMMISSION HAS APPROVED OR DISAPPROVED OF THE SECURITIES OR DETERMINED IF THIS PRESS RELEASE IS TRUTHFUL OR COMPLETE.

**Important Additional Information About the Proposed Transactions Will be Filed with the SEC**

This press release is not a substitute for the registration statement or for any other document that Neoleukin may file with the SEC in connection with the proposed transactions. In connection with the proposed transactions, Neoleukin intends to file relevant materials with the SEC, including a registration statement on Form S-4 that will contain a proxy statement/prospectus of Neoleukin. NEOLEUKIN URGES INVESTORS AND STOCKHOLDERS TO READ THE REGISTRATION STATEMENT, PROXY STATEMENT/PROSPECTUS AND ANY OTHER RELEVANT DOCUMENTS THAT MAY BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THESE DOCUMENTS, CAREFULLY AND IN THEIR ENTIRETY IF AND WHEN THEY BECOME AVAILABLE BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION ABOUT NEOLEUKIN, NEUROGENE, THE PROPOSED TRANSACTIONS AND RELATED MATTERS. Investors and stockholders will be able to obtain free copies of the proxy statement/prospectus and other documents filed by Neoleukin with the SEC (when they become available) through the website maintained by the SEC at www.sec.gov. In addition, investors and stockholders should note that Neoleukin communicates with investors and the public using its website (www.neoleukin.com), the investor relations website (https://investors.neoleukin.com/) where anyone will be able to obtain free copies of the proxy statement/prospectus and other documents filed by Neoleukin with the SEC and stockholders are urged to read the proxy statement/prospectus and the other relevant materials when they become available before making any voting or investment decision with respect to the proposed transactions.

**Participants in the Solicitation**

Neoleukin, Neurogene and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about Neoleukin's directors and executive officers is included in Neoleukin's most recent Annual Report on Form 10-K, including any information incorporated therein by reference, as filed with the SEC, and the proxy statement for Neoleukin's 2023 annual

meeting of stockholders, filed with the SEC on April 27, 2023. Additional information regarding the persons who may be deemed participants in the solicitation of proxies will be included in the proxy statement/prospectus relating to the proposed transaction when it is filed with the SEC. These documents can be obtained free of charge from the sources indicated above.

**Contacts:**

**Neurogene Contacts:**

Investor Relations:
Melissa Forst
Argot Partners
Neurogene@argotpartners.com

Media:
David Rosen
Argot Partners
david.rosen@argotpartners.com

**Neoleukin Contact:**

Investor Relations and Media:
Neoleukin Therapeutics
investors@neoleukin.com

# Neurogene Announces FDA Clearance of IND for NGN-401 Gene Therapy for Children with Rett Syndrome

January 23, 2023

*Planned NGN-401 Phase 1/2 clinical trial to dose female pediatric patients with Rett syndrome in 2023*

*In a comprehensive preclinical program, NGN-401 demonstrated robust therapeutic and safety benefits, delivering MECP2 to key brain regions affected by Rett syndrome*

*NGN-401 utilizes Neurogene's proprietary EXACT technology to regulate transgene expression, and is the first and only gene therapy for Rett syndrome transferring the full-length MECP2 gene*

*NGN-401, manufactured at Neurogene's GMP facility, is Neurogene's second investigational gene therapy product candidate to enter the clinic in the United States*

**NEW YORK, January 23, 2023 –** Neurogene Inc., a clinical-stage 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-401 for the treatment of Rett syndrome.

NGN-401 is the first investigational adeno-associated virus (AAV) gene therapy candidate to be administered to pediatric patients using Neurogene's proprietary Expression Attenuation via Construct Tuning (EXACT) gene regulation technology. EXACT, developed in collaboration with the University of Edinburgh, is a self-contained, transgene regulation technology that can be tuned to deliver a desired level of transgene expression within a narrow range, and is compatible with viral and non-viral delivery platforms. Embedding EXACT technology into NGN-401 is an important advancement in gene therapy for Rett syndrome, specifically because the disorder requires a treatment approach that safely regulates *MECP2* transgene expression without causing toxic effects associated with overexpression.

Intracerebroventricular (ICV) delivery of NGN-401 was evaluated in multiple preclinical models, including the male MECP2 knock out mouse model for efficacy, the female MECP2 mouse model for tolerability, and non-human primates (NHPs) for toxicity. Notably, the efficacy profile for NGN-401 was robust, demonstrating a significant survival benefit with concomitant improvements in Rett syndrome-like phenotypes compared to untreated control animals. Importantly, expression data for NGN-401 demonstrated well-controlled MeCP2 protein levels in key brain regions affected by Rett syndrome, while conventional gene therapy, without EXACT regulation, generated more variable and undesirable higher MeCP2 levels. While comparable doses of NGN-401 in female mice and in NHPs were safe and well-tolerated, in stark contrast, conventional *MECP2* gene therapy without EXACT regulation showed severe toxicity in mice and early signs of toxicity in NHPs.

"Rett syndrome is a particularly challenging disorder for gene therapy because of the requirement to deliver therapeutic levels of *MECP2*, without also triggering significant side effects associated with too much gene expression," said Rachel McMinn, Ph.D., CEO and Founder of Neurogene. "We believe the preclinical profile for NGN-401 is highly compelling, with the strongest results generated to date across multiple animal models. FDA clearance of NGN-401 represents a significant milestone for Neurogene and the Rett syndrome community and underscores our commitment to turn devastating neurological diseases into treatable conditions, and to improve the lives of patients and families impacted by these rare diseases."

"Rett syndrome is a debilitating disease with a devastating impact on children and their families, with no disease-modifying treatments available," said Dr. Bernhard Suter, Assistant Professor of Pediatrics and Neurology at Baylor College of Medicine and neurologist at Texas Children's Hospital. "The upcoming clinical study of NGN-401, which has a mechanism of action aimed at addressing the root cause of disease, offers hope for improving the lives of those suffering from Rett syndrome."

Neurogene recognizes the U.S. Rett syndrome patient advocacy organizations, the Rett Syndrome Research Trust (RSRT) for funding foundational Rett syndrome research at the University of Edinburgh, and the International Rett Syndrome Foundation (IRSF). We appreciate both the RSRT and the Clinical Trial Committee of the IRSF for their extensive collaboration and significant input into the clinical trial design, as well as the large number of caregivers and expert clinicians who provided their unique insights into the key disease manifestations of Rett syndrome. Feedback from all stakeholders, including the FDA, is incorporated into the Phase 1/2 clinical trial design.

IND clearance enables Neurogene to initiate a Phase 1/2 trial to assess the safety, tolerability and efficacy of NGN-401 in female pediatric patients with Rett syndrome. The open-label, single-arm, multi-center clinical trial will evaluate a single dose of NGN-401 delivered using a one-time ICV procedure. More details about the trial design will become available on www.clinicaltrials.gov.

**About NGN-401**
NGN-401 is an adeno-associated virus (AAV) gene therapy investigational product that is the first to deliver the full-length human *MECP2* gene, under the control of Neurogene's EXACT self-contained gene regulation technology. EXACT enables therapeutic levels of the protein MeCP2 while avoiding overexpression related toxicities. NGN-401 has received FDA clearance to be dosed in a one-time administration using the ICV procedure, which Neurogene has shown achieves broad vector distribution to key regions of the brain affected in Rett syndrome. NGN-401 has undergone extensive preclinical study and has demonstrated a robust efficacy profile, coupled with lack of MeCP2 protein related toxicities, even at high doses not intended for human use. NGN-401 is manufactured at Neurogene's GMP manufacturing facility, located in Houston, TX.

**About EXACT**
Expression Attenuation via Construct Tuning (EXACT) is Neurogene's proprietary gene regulation platform technology, developed in collaboration with the University of Edinburgh. EXACT was created to address key limitations of conventional gene therapy, in which cells receiving multiple copies of an AAV therapeutic are "overdosed" with transgene, resulting in transgene related toxicities. EXACT is a self contained gene regulation platform technology that can be tuned to deliver a desired level of transgene expression within a narrow range, thus avoiding transgene toxicities. EXACT is compatible with viral and non-viral delivery platforms, and as delivery methods improve, it may prove to be an important safety tool in gene therapy

designs across disease areas.

**About Rett Syndrome**
Rett syndrome is an X-linked, progressive, neurodevelopmental disorder. Rett syndrome has an estimated incidence of 1 in 10,000 live female births, making it one of the most common genetic causes of developmental and intellectual impairment in females. The incidence in males is currently unknown.

Rett syndrome is caused by mutations in the *MECP2* gene that lead to deficiency of the methyl cytosine binding protein 2 (MeCP2), an important protein responsible for normal function in the brain and other parts of the nervous system. Females with Rett syndrome typically have normal development up until 6-18 months of age. However, females then experience rapid regression of previously acquired milestones including speech, gross and fine motor skills, and develop stereotypical, repetitive hand movements that prevent them from purposeful hand movement or function. Over time females may develop muscle contractures, rigidity, and debilitating scoliosis, along with periods of recurrent seizures, burdensome gastrointestinal abnormalities, breathing abnormalities and cognitive decline.

There are no approved disease-modifying therapies for Rett syndrome. Current treatments for Rett syndrome include symptom management and supportive care.

**About Neurogene Inc.**
The mission of Neurogene is to turn devastating neurological diseases into treatable conditions to improve the lives of patients and families impacted by these rare diseases. Neurogene is developing novel approaches and treatments to address the limitations of conventional gene therapy in CNS disorders. This includes selecting a delivery approach to maximize distribution to target tissues and by designing products to maximize potency and purity for an optimized efficacy and safety profile. The company's proprietary EXACT gene regulation platform technology allows for the delivery of therapeutic levels while limiting transgene toxicity associated with conventional gene therapy. For more information, visit www.neurogene.com.

**Media Contact**
Gwen Fisher
gwen@fishertrentcommunications.com

# Privacy and legal info

This Privacy Notice ("Privacy Notice") was last updated and effective on December 18, 2023.

Neurogene Inc. ("Neurogene") respects the privacy of all individuals who entrust us with their personal data (i.e., information from or about an identified or identifiable person, including information that we can associate with an individual person). This Privacy Notice explains what types of personal data Neurogene may collect from you, how we collect it, how we use it, who we may disclose it to, and how you can manage it. It also describes the policies and practices that we have developed to safeguard personal data and to comply with applicable data protection laws. Please read this Privacy Notice and our Terms of Use carefully.
Our Privacy Notice is not a contract, and it does not create any legal rights or obligations. We may amend this Privacy Notice at any time. When this Privacy Notice is changed, the date of the latest revision will appear at the top of this page.

If you have any questions regarding Neurogene's privacy practices or wish to access or correct personal data Neurogene has collected from you, please contact us using the information below:

**Company name:** Neurogene Inc.
Mailing address: 535 West 24th Street, 5th Floor New York, NY 10011
**Person responsible for privacy inquiries:** Carleen D. Lyken
Senior Counsel
Email address: Privacy@neurogene.com

**Information We May Collect About You**

We may collect, use, store and transfer different categories of personal data about you in electronic (e.g., email, photographs, documents) or paper media. We have grouped them together as follows:

- **Identity Data** includes first name, maiden name, last name, username or similar identifier, marital status, and title.
- **Contact Data** includes address, email address and telephone numbers.
- **Transaction Data** includes details about payments to and from you and other details of products and services you have purchased from us.
- **Technical Data** includes internet protocol (IP) address, your login data, browser type and version, time zone setting and location, browser plug-in types and versions, operating system and platform and other technology on the devices you use to access our Website or intranet.

- **Usage Data** includes information about how you use our Website, intranet, products and services, including: the domain name of the website that allowed you to navigate to our Website, search engines used, the length of time spent on our Website, the pages you looked at on our Website, the frequency of your visits to our Website, and other relevant statistics.
- **Professional, Employment-Related, and Candidacy Information.** When you apply for a job with us, we will collect various of the information categories described above. In addition, we may collect additional identifiers, such as: Social Security Number, driver's license or state identification number, veteran status, race or ethnic origin, gender and other personal and online identifiers; your resume or CV, cover letter, previous work and education experience, and any other professional data collected as part of your employment application and our hiring process; residency, citizenship, or work permit status; and information required for us to comply with laws, including at the direction of law enforcement authorities or court orders.
- **Surveys.** We may contact you to participate in surveys. If you decide to participate, you may be asked to provide certain information which may include personal data.
- **Interactive Features.** We and others who access our Website may collect personal data that you submit or make available through our interactive features (e.g., social media pages). Any information you provide on the public sections of these features will be considered "public", unless otherwise required by applicable law, and is not subject to the privacy protections referenced herein.
- **Additional Information.** Additional information that you provide to us, including through feedback, messages, emails, mail, or otherwise.

**How We Collect Information About You**

**Direct interactions.** You may give us your Identity Data and Contact by filling in forms or by corresponding with us by mail, phone, and e-mail or otherwise. This includes personal data you provide when you contact us through the Website, apply for employment with us.

**Automated interactions.** As you interact with our Website or intranet, we may automatically collect Technical Data and Usage Data about your equipment, browsing actions and patterns, subject to your consent where required. We collect this personal data by using cookies, and other similar technologies. For additional information about how Neurogene uses cookies and similar technologies, see "Cookies and Similar Technologies" section.

**Third Parties (or publicly available sources).** We may receive categories of personal data about you from various third parties and public sources as set out below:

- Technical Data from analytics providers such as Google and search information providers.
- Identity and Contact Data from recruitment agencies.

**How We Use Your Information**

Neurogene does not disclose, give, or sell any personal data you provide to any outside organizations for any reason (other than as described below). We may use any information we collect about you or about your use of the Website for the following purposes:

- **Provide Our Website.** In connection with the operation of our business, as well as to improve the Website or to communicate with you about our business or otherwise in connection with our management of the Website. We may also use information we collect about you for security and protection of personnel, assets, and resources; regulatory compliance and monitoring; and compliance with legal requirements or to defend or pursue legal claims.
- **Website Administration and Development.** We may also monitor traffic patterns and Website usage to maintain, protect, and improve our Website, ensure the technical functions of Our network, and help us develop the design and layout of the Website. We use application logs on your device and our server as well as "cookies" and other tracking technologies for the purposes described below as well as to enhance the functionality of the Website. This information may be stored in files on your device that we access.
- **Changes to Website.** We may also use the information we collect to occasionally notify you about functionality changes to the Website.
- **Evaluate Your Candidacy for a Job with Us.** As a job applicant or recruit, we may use your personal data to evaluate your candidacy for a position with us.
- **Legal Reasons.** To comply with applicable law, respond to valid legal process, participate in legal proceedings, including civil discovery and litigation, protect you, us, and others from unlawful or fraudulent activities, and investigate potential violations of and enforce our policies, including our Terms of Use.
- **With Your Consent.** We may otherwise use your personal data with your consent.

**How We Share Your Information**

We may share the personal data we collect about you as described below:

- **Service Providers.** We may employ independent contractors, consultants, vendors, and suppliers, such as third-party service providers, call centers, mail houses, or any other third party who may need to receive or handle your personal data on our behalf (collectively, "Outside Contractors") in connection with the performance of obligations under this Privacy Notice and to provide specific services and products related to the Website and our business. In the course of providing products or services to us, these Outside Contractors may sometimes have access to your personal data.

- **Subsidiaries and Affiliates.** We may share your personal information with our subsidiaries and affiliates for the purposes described above or as reasonably necessary for our internal administrative and business purposes.
- **Legal Reasons.** We may disclose information we have about you to regulatory authorities, law enforcement agencies, or as required by applicable law.
- **Change of Control Transaction.** We may share personal data with a buyer or other successor in the event of a merger, divestiture, restructuring, reorganization, dissolution or other sale or transfer of some or all of the Company's assets, whether as a going concern or as part of bankruptcy, liquidation or similar proceeding, in which personal data held by the Company about individuals who access our Website is among the assets transferred.
- **With Your Consent.** We may otherwise share your personal information with your consent.

**How We Protect Your Information**

We have put in place appropriate security measures to prevent your personal data from being accidentally lost, used or accessed in an unauthorized way, altered or disclosed. In addition, we limit access to your personal data to those employees, agents, contractors and other third parties who have a business need to know. They will only process your personal data on our instructions and they are subject to a duty of confidentiality.

We have put in place procedures to deal with any suspected personal data breach and will notify you and any applicable regulator of a breach where we are legally required to do so.

**Cookies and Similar Technologies**

Cookies are small files that many websites place on your hard drive that allow those websites to identify you. For example, if you allow a website to remember your login name or password, that website places a cookie on your computer.

We use cookies for technical purposes, statistical purposes and advertising purposes. Indeed, subject to your prior consent, we may place cookies on your computer to allow us to identify you during future visits to our Website. We may use cookies to measure web traffic and to customize your visit. If you consented to our use of cookies but later wish to opt out, you can change the browser settings, at any time, in order to block the cookies. If you block cookies, you may not be able to use certain features or functions of this Website, or this Website may not operate in optimal mode.

A beacon is an electronic tracking mechanism that usually consists of a single-pixel image. It can be embedded in a web page or in an email to transmit information, which could include personal data. For example, it allows an email sender to determine whether a particular email has been opened.

We may also use Google Analytics or other similar software or services, with your prior consent, to gather certain information in connection with the use of the Website. When we use Google Analytics, your web browser automatically sends certain information to Google about your use of the Website, such as the web address of the page you're visiting and your IP address. You may opt-out by visiting this link. For more information on how Google Analytics collects, protects, uses, and shares your data, click here.

**How We Respond to Do Not Track Signals**

Some web browsers may transmit "do-not-track" signals to websites with which the browser communicates. Websites linked to this Privacy Notice do not currently respond to these "do-not-track" signals.

**Choices You Have About Our Use of Your Information**

You can write to us at any time to obtain a copy of your information and to have any inaccuracies corrected or if you no longer wish to be registered on the Website. Where appropriate, you may have your personal data erased, rectified, amended, or completed. In order to contact us regarding your information, you may send us an email at privacy@neurogene.com.

**Links to Third Party Websites**

The Website may contain links to other websites. These third-party sites may send their own cookies to you to collect data or solicit personal data. Please be aware that other sites are not subject to this Privacy Notice and Neurogene is not responsible for the privacy practices of these other sites. We encourage you to be aware when you leave our site and to read the privacy statements of each and every website that collects personal data. This Privacy Notice applies solely to information collected by the Website.

**Children's Privacy**

The Website is not directed to children under the age of 13. We do not knowingly collect personal data from children under the age of 13. If an individual identifies themselves as a child under the age of 13, we will not collect, store, or use any personal data. If we receive personal data that we discover was provided by a child under the age of 13, we will promptly destroy such information.

**Updating this Privacy Notice**

From time to time, we may change our privacy practices.

We will post any updates to this Privacy Notice on our Website, with a "last updated and effective" date at the top of this document. Please check this page for updates.

**SUPPLEMENTAL UK PRIVACY NOTICE**

For purposes of United Kingdom ("UK") data protection laws, Neurogene Inc. is the data controller, i.e., the company responsible for controlling the processing of personal data covered by this Privacy Notice.

The UK Data Protection Act 2018 (the "DPA 2018") and the UK GDPR (as defined in section 3(10) (as supplemented by section 205(4)) of the DPA 2018) require Neurogene as the data controller to provide additional and different information about its data processing practices to data subjects in the UK. If you are a data subject within the UK, this Supplemental UK Privacy Notice applies to you in addition to the provisions above.

HOW WE USE YOUR PERSONAL DATA

We will only use your personal data when the law allows us to do so. Most commonly, we will use your personal data in the following circumstances:

- We need to perform the contract we are about to enter into or have entered into with you.
- It is necessary for our legitimate interests (or those of a third party) and your interests and fundamental rights do not override those interests.
- We need to comply with a legal or regulatory obligation.

We may also rely on consent as a legal basis for processing your personal data in certain circumstances, like sending direct marketing communications to you via email or text message. You have the right to withdraw consent at any time by contacting us.

We have set out below, in a table format, a description of many of the ways we plan to use your personal data, and which of the legal bases we rely on to do so. We have also identified what our legitimate interests are where appropriate.

Note that we may process your personal data for a different lawful basis for each purpose for which we are using your data. Please contact us if you need additional details about the specific legal ground we are relying on to process your personal data.

CHANGE OF PURPOSE

We will only use your personal data for the purposes for which we collected it, unless we reasonably consider that we need to use it for another reason and that reason is compatible with the original purpose.

If we need to use your personal data for an unrelated purpose, we will notify you and we will explain the new legal basis. If such change of purpose requires your consent, you will have the choice to consent as to whether or not we may use your personal data in a different manner.

Please note that we may process your personal data without your knowledge or consent, in compliance with the above rules, where this is required or permitted by law.

INTERNATIONAL TRANSFERS OF PERSONAL DATA

We are based outside the UK, so the processing of your personal data may involve a transfer of data outside the UK. Information on how to contact our privacy officer can be found at the introduction of this Notice.

Whenever we transfer your personal data out of the UK, we ensure a similar degree of protection is afforded to it by ensuring at least one of the following safeguards is implemented:

- We will only transfer your personal data to countries that have been deemed to provide an adequate level of protection for personal data by the UK Secretary of State.
- Regarding transfers to a country whose legislation has not been recognized by the UK Secretary of State as having an adequate level of protection (for instance, transfers to the US), we are required to incorporate the International Data Transfer Agreement in agreements in order to provide similar protection to personal data shared within the UK. In this regard, please note that we will provide you with a copy of applicable safeguards upon request, at the contact details specified at the end of this Notice.

HOW LONG WE RETAIN YOUR PERSONAL DATA

We will only retain your personal data for as long as necessary to fulfil the purposes we collected it for, including for the purposes of satisfying any legal, accounting, or reporting requirements.

To determine the appropriate retention period for personal data, we consider the amount, nature, and sensitivity of the personal data, the potential risk of harm from unauthorized use or disclosure of your personal data, the purposes for which we process your personal data and whether we can achieve those purposes through other means, and the applicable legal requirements.

With respect to the services and products provided to you, we will keep your personal data during the period of our contractual relationship extended by the applicable limitation period. With regard to marketing communications with prospects, we will keep your personal data for three (3) years after the last correspondence with us.

With respect to the processing carried out in order to protect or defend your and our rights, property and security, we will keep your personal data for the time of the relevant dispute or statute limitation period.

As to the processing carried out in order to comply with a legal requirement, we will keep your personal data for the duration of such obligation.

YOUR DATA PROTECTION RIGHTS

Under certain circumstances, visitors from within the UK have the following data protection rights:

- access to your personal data.
- correction of your personal data.
- erasure of your personal data.
- object to processing of your personal data.
- restrict of processing your personal data.
- transfer of your personal data (data portability).
- withdraw consent to any consent that you have previously given.

If you wish to exercise any of the rights set out above, please contact our privacy officer. You can also contact the Information Commissioner's Office at this link to make a complaint.

VeraSafe has been appointed as Neurogene's representative in the UK for data protection matters, pursuant to Article 27 of the UK GDPR. VeraSafe can be contacted in addition to or instead of Neurogene's privacy officer, only on matters related to the processing of personal data.

To make such an inquiry, please contact VeraSafe using this contact form: https://verasafe.com/public-resources/contact-data-protection-representative or via telephone at: +44 (20) 4532 2003.
Alternatively, VeraSafe can be contacted at:
**VeraSafe United Kingdom Ltd.** 37 Albert Embankment London SE1 7TL United Kingdom.
You will not have to pay a fee to access your personal data (or to exercise any of the other rights). However, we may charge a reasonable fee or refuse to comply with your request if it is clearly unfounded, repetitive or excessive.

We may need to request specific information from you to help us confirm your identity and ensure your right to access your personal data (or to exercise any of your other rights). This is a security measure to ensure that personal data is not disclosed to any person who has no right to receive it. We may also contact you to ask you for further information in relation to your request to speed up our response.

We try to respond to all legitimate requests within one month. Occasionally it may take us longer than a month if your request is particularly complex or you have made a number of requests. In this case, we will notify you and keep you updated.

# Professional:

## Overview

Neurogene is accelerating development of new genetic medicines to people with devastating neurological diseases and their families. To do this, we are working with experts across the globe to advance a broad pipeline of programs to treat the underlying cause of serious neurological disorders and thereby address the overwhelming need for new therapies.

Although the human genome was sequenced for the first time nearly 20 years ago, the true genomics revolution is taking place now, providing us with the ability to radically alter rare, genetic disorders. At Neurogene, we are working to provide medicines to improve the lives of neurologically-impaired and developmentally-delayed children and their families.

We are building a team of passionate, driven individuals who envision a world in which all families have access to genetic medicines, even if the disorder is exceedingly rare.

To learn more, please visit http://www.neurogene.com/careers/.

Website

http://www.neurogene.com

Industry

Biotechnology

Company size

51-200 employees

85 associated members LinkedIn members who've listed Neurogene Inc. as their current workplace on their profile.

Headquarters

New York, New York

Founded

2018