

# NATIONAL NEUTROPENIA NETWORK

FALL 2023  
NEWSLETTER



## FAMILY RETREAT, JULY 14-16, CINCINNATI, OHIO

The NNN was excited to host the first in person family retreat since 2018! The weekend was a huge success with 36 registered kids for Kids Camp, 72 registered adults, 7 physicians, a representative from [X4 Pharmaceuticals](#), 2 scientists from [EmendoBio](#), 4 service dogs, and 19 other volunteers and professionals in attendance ~ all representing 21 states and 3 countries outside the US. The weekend consisted of community building, hearing from Voices of Experience, presentations from top neutropenia specialists, introductions to current research studies and understanding the research trial process, sharing of personal stories and building friendships, and entertainment from a local mascot and rising country music singer. The beloved Kids Camp, sponsored by the Ella Jewell Foundation, helped kids forge impactful relationships with others in the community.

The NNN recognized Dr. David Dale for his lifetime commitment and achievement to neutropenia care, management, and technology advancement. Dr. Dale's leadership in the neutropenia community is second to none. We thank Dr. Dale for attending the Retreat this year, alongside Dr. Peter Newburger, Dr. Akiko Shimamura, Dr. Kasiani Myers, Dr. Jane Koo and Dr. Zahra Hudda.

We want to thank our Platinum Sponsor, X4 Pharmaceuticals, as well as our supporting sponsors, [EveryLife Foundation](#) and the [Ella Jewell Foundation](#) for making the Retreat weekend the best one yet! Save the date for Summer 2025 when we will host the next in person Retreat. The NNN will occasionally host webinars throughout the year--stay connected with the most up to date information via the NNN website [neutropenianet.org](http://neutropenianet.org).

*"We learned so much and in this year of uncertainty and anxiety, we never felt more prepared to stand up and fight for the best care. We now have so many tools at our disposal and a plan or action for certain diagnosis! We are forever grateful for this event, the knowledge, and the connections we have made!" ~ Parents of newly diagnosed infants*

*"This experience changed my life forever! When I started this journey I was alone and felt lost. Now, we are all together like a huge family. We may share a terrible disease, but more important, we share our strength and that's all we need to live our lives in the best way possible!" ~ Adult with neutropenia*

**[Links to the recorded Retreat presentation sessions can be found here.](#)**

**[Links to the Retreat weekends Powerpoint slide presentations can be found here.](#)**

**[Links to the Retreat Google photo galleries can be found here:](#)**  
[professional](#)   [attendees](#)

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## KEEPING YOU UP TO DATE

### A MESSAGE FROM DR. DALE:

*“As Audrey Anna Bolyard and I discussed with many of you at the recent NNN meeting in Cincinnati, our research laboratory at the University of Washington continues to study neutropenia due to ELANE mutations and search for other causes for chronic neutropenia. Currently we are conducting a study using sputum samples from individuals with known ELANE mutations to confirm the mutation and sequence the DNA “up and down stream” from the mutation. We are doing this research to help with developing a way to edit DNA and very selectively eliminate the mutant gene that causes ELANE neutropenia. With your consent, we will send you a container for a sputum sample from which we can harvest enough DNA to do this analysis.*

*If you believe your neutropenia is from childhood and you do not know if you have a mutation causing it, we are interested in hearing from you also. We will try to help you to get DNA testing to see if you have a genetic mutation causing your neutropenia.*

*Anyone with interest should contact Audrey Anna Bolyard at [bolyard@uw.edu](mailto:bolyard@uw.edu), Jamie Leung [owlie@uw.edu](mailto:owlie@uw.edu), or me [dcdale@uw.edu](mailto:dcdale@uw.edu), Or please just call my office 206-543-7215.”*

*- David C. Dale, MD*

Anyone who wishes to learn more about Dr. Dale’s collaboration with **EmendoBio** can go to [EmendoBio’s page for patients](#) or the [recorded \[EmendoBio\] presentation](#) (both are also available on the Retreat page).

### X4 PHARMACEUTICALS

X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company and a leader in the discovery and development of novel therapies for the treatment of diseases of the immune system, with a focus on rare diseases and those with limited treatment options. Their lead clinical candidate, mavoxifafor, is being developed as a once-daily, oral therapy. X4 believes that successfully developing mavoxifafor and providing a new therapeutic option for individuals diagnosed with chronic neutropenic disorders and WHIM syndrome has the potential to revolutionize the treatment landscape, which is currently only served by injectable therapies often associated with treatment-limiting side effects.

X4 is now enrolling patients into a study for people living with chronic neutropenia. To learn more about the study, [click here](#). To enroll in the study, [click here](#).

**Opportunity to share your experiences!** We have learned of an opportunity for up to 8 people living with chronic neutropenia to participate in one-on-one virtual patient advisor meeting. X4 Pharmaceuticals would like to hear from people in our community about their experiences. Selected participants will be compensated for their time.

Must be diagnosed with chronic neutropenia (idiopathic, primary autoimmune, or congenital), currently on G-CSF, 18+, located in United States. Interested in participating in this virtual meeting, [please complete this brief interest form by Friday, October 13:](#)

*Providing feedback through this project will not impact your future ability to participate in X4 clinical trials. Individuals currently enrolled in a trial are not eligible to serve as an advisor. Although space is limited, you’re welcome to share the link with others you think may be interested.*

### SCNIR (SEVERE CHRONIC NEUTROPENIA INTERNATIONAL REGISTRY)

The SCNIR has moved to Boston because its major financial support comes from an NIH grant that was renewed this time by Drs. Newburger and Shimamura in Boston. Dr. David Dale and Audrey Anna Bolyard, who founded the registry in the US, will remain intimately involved in its operation and patient/family relations. [Click here](#) for detailed information on the SCNIR.

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## COMING SOON ...

The NNN will be partnering with a company called **Probably Genetic** whose mission is to help rare genetic disease patients find out what they have. Keep an eye on our website [www.neutropenianet.org](http://www.neutropenianet.org) for a link to connect with their Symptom Checker, to see if you qualify for free genetic testing. You can learn more about **Probably Genetic** at [www.probablygenetic.com](http://www.probablygenetic.com).

## THANK YOU TO OUR VOLUNTEERS!!!

The framework of the Network runs on volunteers!!! From our Board members, to Junior and Senior Ambassadors, to Peer Support Volunteers, to story writers, to IT specific tasks, to community outreach, to Retreat volunteers and beyond---we couldn't do it without you! Thank you for giving of your time and talents to help make an impact in the community! We would love to have you join our team! [Click here to submit a volunteer interest form.](#)

Our **Senior and Junior Ambassadors** do a phenomenal job representing the neutropenia community. Click on the links above for their stories and how they have become and plan to become involved with the NNN.

Remember when you or your loved one was first diagnosed? All of those feelings early on in diagnosis can be overwhelming. Our **Peer Support Volunteers** connect with those seeking support and are an invaluable asset to the Network. If you would like to become a member of the Peer Support Program, [click here](#) to complete the application.

### RESOURCES

- [Request peer support](#)
- [Emergency wallet card](#)
- [NNN Facebook](#)
- [NNN Twitter](#)
- [NNN Instagram](#)
- [Contact for SCNIR](#)

### THE NNN TEAM

- Stephanie Long, Executive Director
- Katie loader, Accountant
- Gretchen Long, Board Chair
- Tammy Loader, Secretary
- Robert McGuinness, Treasurer
- Tanya Lakhia, Board Member
- Vanessa Ploederl, Board Member