

# Spring 2019 Newsletter

## Quality of Life Survey

The National Neutropenia Network in association with primary immunodeficiency researchers are performing a study to learn more about the quality of life and physical/mental/social health of adult and pediatric neutrophil/phagocyte immunodeficiency patients. To get information, we'd like patients and/or their primary caregiver to answer a survey. We expect it to take about 30-40 minutes to complete the survey. Of note, children under the age of 5 years old are not eligible for the study.

Answering this survey is voluntary. You don't have to answer it if you'd rather not. You can skip any questions that you don't want to answer, whatever the reason, and you don't have to tell us why. Choosing not to answer our survey won't affect the medical care you might receive in the future. It's possible that some of the questions may make you feel uncomfortable. If a question makes you uncomfortable, you can just skip it and go to the next question.

All information obtained from this survey will be kept confidential and your survey responses will be completely anonymous. No one, including members of our study team, will know which subjects gave which answers. Answering our survey won't benefit you directly but we hope what we learn from our study will help patients diagnosed with neutrophil conditions and allow medical teams to better care for these patients.

If you are willing to participate in our study, please use the appropriate link below to be taken to a secure web-page to complete a form of consent and begin to answer study questions.

[Patients aged 18 years or older](#)

[Patients aged 5-17.9 years old](#)

Thank you for your time.

## Helpful Resources

Emergency Room Card  
[English ER card \(PDF\)](#)  
[Español ER tarjeta \(PDF\)](#)

[ANC Calculator](#)

[E-Cards: submit & send](#)

Information on types of neutropenia, management, and more at:  
[The Severe Chronic Neutropenia International Registry](#)

[Ella Jewel Foundation](#)  
[Cohen Syndrome Association](#)

Ways to Support the NNN  
[Round Up App](#)  
[Shop for T-shirts, sweatshirts, & more](#)  
[Giving Assistant-Shop & Save](#)

Endings & New Beginnings:

Megan Theoret

Megan Theoret has been our Website Host and Developer for the past 5 years. She has recently made the decision to close her business and move on to other dreams.

Megan states, "I like being part of a non-profit, mission-driven work." Megan lives in Boulder, Colorado and will be working part-time until she figures out the next chapter in her life.

Thank you, Megan, for your creativity, web-page fixes, and hard work! You will be missed and we wish you the best.

## 4 WHIM Clinical Trail

### Phase 3

X4 Pharmaceuticals (Cambridge, MA) is planning to start a Phase 3 clinical study within the next few months, entitled "A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Multicenter Trial of mavorixafor (X4P-001) in Patients with WHIM Syndrome with Open-Label Extension"

WHIM (**W**arts, **H**ypogammaglobulinemia, **I**nfections and **M**yelokathexis) is a rare autosomal dominant primary immunodeficiency disease caused by gain-of-function mutations in the chemokine receptor CXCR4. CXCR4 mutations induce increased leukocyte homing and retention in the bone marrow resulting in severe chronic neutropenia and lymphopenia.

X4 Pharmaceuticals is planning to sponsor a 52-week global Phase 3 study to evaluate the safety and efficacy of mavorixafor, a once-daily oral CXCR4 antagonist, in genetically confirmed WHIM patients who are aged 12 years or older. Mavorixafor is a first-in-class, oral, small molecule

## Doctor's Corner

Question: I would like to travel and as an individual with neutropenia, can I have the vaccines and is there anything else I can do to protect myself to stay healthy when traveling?

Answer:

If you have neutropenia, it is important to have a health care professional help evaluate which vaccines are safe when neutrophil counts are low. Individuals with Neutropenia should not have problems with inactivated vaccines- possibly very mild symptoms with attenuated vaccines but not the disease. Flu shots are recommended.

There are four types of vaccines currently available:

- **Live virus vaccines** use the weakened (attenuated) form of the virus. The measles, mumps, and rubella (MMR)

antagonist of the CXCR4 chemokine receptor that has demonstrated a favorable safety profile and dose-dependent increases in ANC and ALC in a Phase 2 study. Trial subjects will participate in a one-year trial with an opportunity to continue in the subsequent open label extension. Travel support to study sites will be provided.

To support confirming the diagnosis of WHIM, X4 Pharmaceuticals will offer genetic testing at no charge to patients meeting eligibility criteria suggestive for the disease. For information and support for genetic confirmation of your patients with suspected WHIM, please contact [patientinfo@x4pharma.com](mailto:patientinfo@x4pharma.com)

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### Successful Bar Mitzvah!

Sadie wanted to do something for her brother, Jackson, who has neutropenia. She wanted to make a difference. So, she decided to host a fundraiser and have the proceeds benefit the National Neutropenia Network and those with neutropenia. During the Bar Mitzvah, 18 donations were given, totaling \$625. Way to go, Sadie & Jackson. Thank you from all of us for your hard work and support.

### Shop & Support the NNN!

You care about our cause; and we care about YOU! That's why we've teamed up with Giving Assistant to make it easier to donate.

Simply sign up, select our cause, and shop at over 3,000+ online stores. You'll save money and earn cash back on your everyday purchases, all while making a difference.

Shop by clicking on the 'Giving Assistant' Logo! Happy Shopping!

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### Give Today

The National Neutropenia Network depends on support from the friends and families of those whose lives have been forever changed and challenged by this rare disease. Thank you for your support.

vaccine and the varicella (chickenpox) vaccine are examples.

- **Killed (inactivated) vaccines** are made from a protein or other small pieces taken from a virus or bacteria. The whooping cough (pertussis) vaccine is an example.
- **Toxoid vaccines** contain a toxin or chemical made by the bacteria or virus. They make you immune to the harmful effects of the infection, instead of to the infection itself. Examples are the diphtheria and tetanus vaccines.
- **Biosynthetic vaccines** contain manmade substances that are very similar to pieces of the virus or bacteria. The Hepatitis B vaccine is an example.

Other precautions you can take when traveling: wash hands, wear a mask on the plane, bring wipes to clean areas in hotel rooms, drink bottle water- using it to clean teeth also, as local water can have different levels of bacteria or yeast that an individual with neutropenia can find troublesome. In addition, wash all fresh food and do not eat fresh fruit or vegetables that can not be peeled- give up lettuce while traveling- cooked vegetables and fruit are okay.

Thank to Audrey Anna Bolyard, RN, B.S., for providing our answer. Audrey Anna Bolyard is the Clinical Manager with the Severe Chronic Neutropenia International Registry at the University of Washington-Seattle.



### Thanks for Subscribing

The team at [National Neutropenia Network](#) wants to extend a hearty 'thank you' to all of our wonderful readers. You've helped make our community shine, and we want to keep that going! For more information on our mission and how you can help visit [our website](#).