

Spring 2022 Newsletter: Special Research Edition

New Study Open: Phase 1b Mavorixafor



X4 Pharmaceuticals has a clinical trial for people living with congenital, cyclical and idiopathic neutropenia. Clinical trials are important to help advance research and make new therapies available. Clinical trial volunteers help continue this research and bring new treatment options to communities. This trial involves 1 dose of oral capsules with 24 hours of monitoring that can be in person or remote (not in the doctor's office).

Early data of this study (shared at ASH last year) showed that a single dose of the study drug, mavorixafor, increased ANC in people with chronic neutropenia.

[Click here to view more information about the study, what it involves, and who can join.](#)

We are eager to reach those with congenital and cyclical neutropenia and hope to include as many people with different types of neutropenia and different genetic variants as possible to make sure future trials are also available to a wide cross section of people. One concern is that if we don't get enough data from people with different subsets of neutropenia, we won't be able to include them in future trials.

If you are interested in learning more about the trial and are interested to see if you qualify, please [email](#) X4.



The phase 2 study tested a potential oral treatment for WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis) syndrome. WHIM syndrome is caused by mutations in the CXCR4 gene and is a rare primary immunodeficiency.

There are no existing treatments that specifically target WHIM syndrome, so people with WHIM syndrome may only have treatments available that address symptoms like neutropenia or low immunoglobulins.

The results of this study helped researchers design a phase 3 study of mavorixafor in patients with WHIM syndrome.

What were the results of the study?

- All participants in the study tolerated mavorixafor. The drug did not cause any serious treatment-related side effects.
- Participants treated with mavorixafor for at least 6 months experienced fewer infections and warts.
- At the higher doses of 300mg and 400mg, participants experienced:
 - Fewer bacterial infections
 - 75% average decrease in warts
 - Improved total white blood cells, neutrophils (ANC), and lymphocytes (ALC) — signs of a strengthened immune response.

[Click here to read full flyer about the results.](#)

Learn more about WHIM syndrome and X4 Pharmaceuticals research at: www.x4pharma.com.

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](#).

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Quarterly Newsletter

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