WHIM SYNDROME PHASE 2 STUDY:
POTENTIAL THERAPY MAY LOWER INFECTIONS AND WARTS

X4 PHARMACEUTICALS

Early research shows mavorixafor may be a promising therapy, and results helped to develop the phase 3 study design

What was the study about?

This 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 was the goal of the study?

Researchers wanted to find out if the oral drug mavorixafor can safely reduce bacterial infections and warts in people with WHIM syndrome. Researchers also wanted to know if mavorixafor is safe for people with WHIM syndrome.

What did we learn and what were the results of the study?

Researchers learned that mavorixafor may help people with WHIM syndrome by targeting the way the disease works.

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.

LEARN MORE ABOUT THE STUDY →

What happened in the study?

Participants received different doses of mavorixafor, an investigational drug taken once daily by mouth. Researchers looked at biomarkers, or signals in the blood, to see if the participants showed stronger immune responses while taking this drug. They also checked to see how the body recognizes and defended itself against bacteria depended on the amount of the drug given.

There were **8 adult participants** with WHIM syndrome at **2 study sites in Australia and the U.S.**

Participants received increasing doses of mavorixafor, based on blood tests of each of their absolute lymphocyte counts (ALC) and absolute neutrophil counts (ANC). **Not all participants received all dose levels of the drug.**

The increase in doses took place over 25 to 52 weeks, with a maximum dose of 400mg.

Researchers measured infection rates at each dose and compared them to rates before the study took place.

Researchers counted the number of warts on the hands and feet of participants to see if there were any changes.

Will there be more studies on this treatment?

The results of this study helped researchers design a phase 3 study of mavorixafor in people with WHIM syndrome. This study will test a 400mg dose, looking at how long ANC counts can remain above a desired level in participants. The study will also look at the number of infections and warts participants experience.

Where can I find more information?

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

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