

X4P-001-RD in WHIM

X4P-001-RD is being developed for use as a lifelong treatment for patients with WHIM and other primary immunodeficiencies. X4P-001-RD is being studied in a Phase 2/3 trial in patients with WHIM syndrome.

Mutations in the CXCR4 receptor gene have been identified as the underlying cause of WHIM syndrome. In WHIM patients, CXCR4 signaling is overactive and results in aberrant trafficking of specific immune cells that are critical for proper immune system functions. X4P-001-RD is designed to normalize the signaling from the mutant CXCR4 receptor to promote normal release of neutrophils and leukocytes, thereby restoring normal immune surveillance and normal immune function.

X4P-001-RD may provide a new treatment approach focused on the underlying disease process of WHIM, whereas current therapy is limited to treatment of acute infections with antibiotics or prevention through the use of intravenous immunoglobulins.