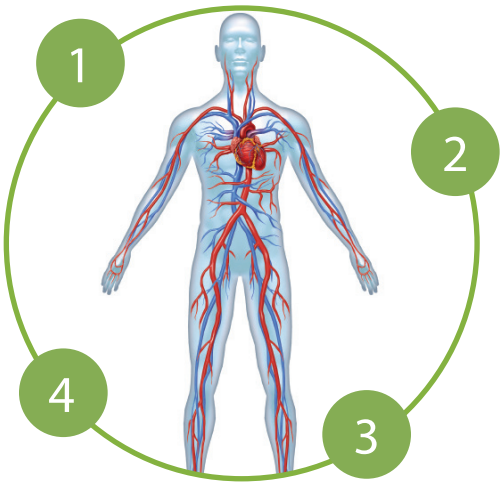
What is autologous ex vivo gene therapy?

Autologous ex vivo gene therapy is being investigated for the treatment of patients with monogenic diseases

Autologous ex vivo gene therapy introduces a specific functional gene into a patient's own cells[1]

HSCs are isolated from a patient's peripheral blood or bone marrow, genetically modified ex vivo, and then reintroduced back into the patient, thereby introducing a functional gene.[1]

Step 1: Stem cells are taken from the patient, for example via leukapheresis from the peripheral blood or via a bone marrow extract.[1]



Step 2: Stem cells are genetically modified outside of the body (ex vivo) using a viral vector carrying a normal copy of the gene.[1]

Step 4: Genetically modified stem cells are engrafted according to the patient's disease condition.[1]

Step 3: Genetically modified stem cells are given back to the patient, for example via intravenous infusion.[1]

Autologous ex vivo gene therapy is currently under clinical development for the treatment of monogenic disorders including primary immunodeficiencies, neurometabolic disorders, and hemoglobinopathies.[1]

Orchard therapeutics

Reference: 1. Leboulch P. Gene therapy: primed for take-off. Nature. 2013;500(7462):280-282. doi:10.1038/500280a.

Orchard therapeutics™