A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-Linked Chronic Granulomatous Disease

Disease Area: X-linked Chronic Granulomatous Disease
Investigator: Donald Kohn
Institution: University of California, Los Angeles
CIRM Grant: CLIN2-08231
Award Value: $7,083,364
Trial Sponsor: University of California, Los Angeles
Trial Stage: Phase 1/2
Trial Status: Active, not recruiting
Targeted Enrollment: 10
ClinicalTrials.gov ID: NCT02234934

Details:
X-linked Chronic Granulomatous Disease (X-CGD) is a rare immune disorder that prevents white blood cells from killing foreign invaders. This results in severe, recurrent infections that can impact quality and length of a patient’s life. X-CGD is usually diagnosed before age 5, but without treatment, children die before age 10. A team at UCLA is using the patient’s own genetically modified blood stem cells to create a new blood supply and a healthy immune system, with the aim of curing patients with this disease.

Design:
X-linked Chronic Granulomatous Disease.

Goal:
Primary: Safety and Efficacy. Secondary: Restoration of immune function

Updates:
Enrolling. Early evidence of clinical efficacy.

Source URL: https://www.cirm.ca.gov/clinical-trial/phase-iii-non-randomized-multicenter-open-label-study-g1xcgd-lentiviral-vector