

20.05.2021

Final report - EudraCT-No. 2012-001725-26

Sponsor's protocol code number: X-CGD-Version 2

Full title of trial: A Phase I/II Gene Therapy trial for X-CGD with a SIN gamma retroviral vector

Sponsor: Dekan des Fachbereichs Medizin der Goethe Universität, represented by the Coordinating Investigator

This clinical trial was intended to treat adult male individuals with severe X-linked chronic granulomatous disease (X-CGD) with ex-vivo gene-modified autologous hematopoietic stem cells after myeloablative chemotherapy. A SIN-gammaretroviral vector had been developed for the ex-vivo gene transfer. The study has been initiated in October 2013. In general, recruitment of adult X-CGD patients suitable for this trial was challenging, and frequently hampered by the general health condition of the candidates that did not allow myeloablative chemotherapy. The inclusion of one patient considered for treatment in late 2013 had to be postponed due to an acute infection. However, in 2014 a lentiviral gene-therapy approach for the same cohort of patients, that promised enhanced efficacy and lower risk of genotoxicity became available (EudraCT: 2012-000242-35). No patient has been treated within the trial.