

Wednesday, March 24, 2021
11:00am - 12:00pm PST



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“Platelet-specific gene therapy for hemophilia A”

Hemophilia A is a genetic bleeding disorder resulting from a FVIII deficiency. Currently, protein replacement therapy is effective, but it is expensive and requires frequent access vessels for infusion. Furthermore, 30% of patients will develop inhibitory antibodies (inhibitors), rendering routine protein infusion useless. Gene therapy is an attractive alternative for hemophilia treatment as it may provide a cure of the disease if successful. We have developed a platelet-specific gene therapy protocol in which FVIII is expressed and stored in platelet α -granules. Our studies showed that platelet gene therapy not only corrects the bleeding phenotype but also induces antigen-specific immune tolerance in hemophilia A mice even with pre-existing anti-FVIII immunity. Our further studies showed that lentiviral gene delivery to hematopoietic stem cells could effectively introduce FVIII expression in human platelets and rescue the hemophilic phenotype in xeno-transplanted animals. Thus, we conclude that platelet gene therapy is a promising approach for hemophilia A treatment. Currently, this protocol is in Phase 1 clinical trial.