

Autologous hematopoietic stem cell (HSC)-targeted gene therapy provides a one-time cure for various genetic diseases including sickle cell disease (SCD) and  $\beta$ -thalassemia. In HSC gene-addition therapy, patient CD34+ HSCs are genetically modified by adding a therapeutic  $\beta$ -globin gene with lentiviral transduction, followed by autologous transplantation. Alternatively, novel gene-editing therapies allow for the correction of the mutated  $\beta$ -globin gene, instead of addition.