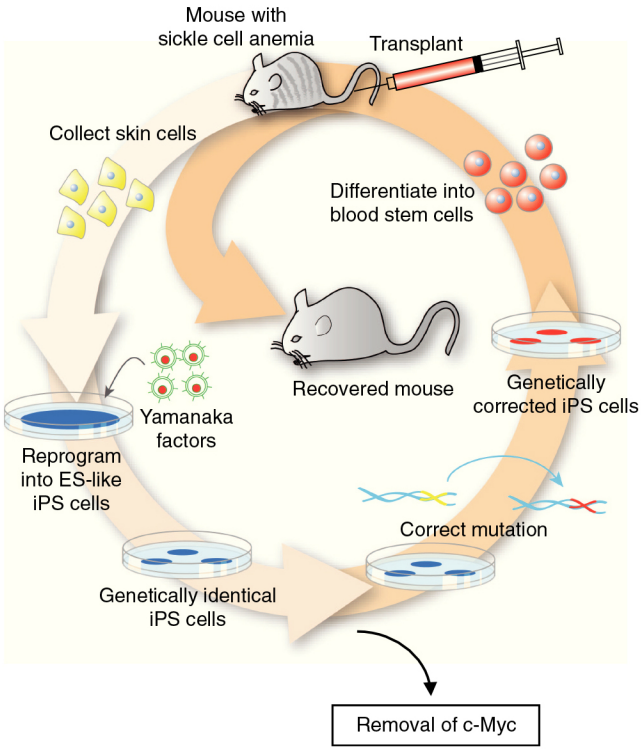

Figure 11. Proof of concept for cell therapy using iPSCs in a humanized model of sickle cell anemia. Transgenic mice carrying the human α-globin gene and the anemia-causing β-globin variant develop disease that resembles human sickle cell anemia. Skin cells were reprogrammed to iPSCs by the four Yamanaka factors and c-Myc was removed by Cre-mediated excision. Homologous recombination was used to correct the mutation in the defective β-globin gene; the corrected iPSCs were differentiated into hematopoietic stem cells and transplanted into the mutant mice. The cells engrafted and generated normal red blood cells curing the anemia.

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