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Treatment of sickle cell anemia mouse model with iPS cells generated from autologous skin.

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It has recently been demonstrated that mouse and human fibroblasts can be reprogrammed into an embryonic stem cell-like state by introducing combinations of four transcription factors. However, the therapeutic potential of such induced pluripotent stem (iPS) cells remained undefined. By using a humanized sickle cell anemia mouse model, we show that mice can be rescued after transplantation with hematopoietic progenitors obtained in vitro from autologous iPS cells. This was achieved after correction of the human sickle hemoglobin allele by gene-specific targeting. Our results provide proof of principle for using transcription factor-induced reprogramming combined with gene and cell therapy for disease treatment in mice. The problems associated with using retroviruses and oncogenes for reprogramming need to be resolved before iPS cells can be considered for human therapy.

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