healing the vascular and tissue damage associated with stroke and myocardial infarction. These data are controversial, and the applicability of a stem cell approach to nonhematopoietic conditions remains experimental. However, reprogramming technology offers the potential for using readily obtained hematopoietic cells as a source for cells with other capabilities. Active areas of investigation are to use reprogrammed cells to generate mature lymphoid cells for immuno-oncology applications or red cells and platelets to overcome dependency on blood donors.

STEM CELLS AS TARGETS OF GENE THERAPY

Tools to alter gene sequence, expression, and regulation are becoming increasingly feasible. The hematopoietic stem cell is a target for a wide range of interventions. Lentiviral, retroviral, and adenoviral vectors are being used to replace defective genes (e.g., in primary immunodeficiency diseases). Antisense technology is being applied to block gene expression (e.g., blocking the Bcl11a repression of fetal globin expression in sickle cell disease and thalassemia). CRISPR/Cas technology is being applied to repair abnormal gene sequences. Precision genetic manipulations are expanding, and the hematopoietic system is central to it.

In sum, the stem cell has tremendous healing capacity and is essential for life. However, if dysregulated, it can threaten the life it maintains. Understanding how stem cells function, the signals that modify their behavior, and the tissue niches that modulate stem cell responses to injury and disease is critical for more effectively developing stem cell–based medicines.

■ FURTHER READING

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