

Small molecule-based approaches to adult stem cell therapies.

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Public Summary:

Stem cell-based therapies show promise for the treatment of a broad range of human diseases, including neurodegenerative, autoimmune, cardiovascular, and musculoskeletal diseases. There have been two major types of approach to development of stem cell therapies: 1. cell replacement therapies, which involve transplantation of stem cell-derived cells, and 2. using drug-like small molecules to stimulate the somatic stem cells that exist in many tissues. Here we review examples of the use of chemical regulation of endogenous somatic stem cells that are yielding new biological insights and that may ultimately lead to innovative new medicines.

Scientific Abstract:

There is considerable interest in the development of stem cell-based strategies for the treatment of a broad range of human diseases, including neurodegenerative, autoimmune, cardiovascular, and musculoskeletal diseases. To date, such regenerative approaches have focused largely on the development of cell transplantation therapies using cells derived from pluripotent embryonic stem cells (ESCs). Although there have been exciting preliminary reports describing the efficacy of ESC-derived replacement therapies, approaches involving ex vivo manipulated ESCs are hindered by issues of mutation, immune rejection, and ethical controversy. An alternative approach involves direct in vivo modulation or ex vivo expansion of endogenous adult stem cell populations using drug-like small molecules. Here we describe chemical approaches to the regulation of somatic stem cell biology that are yielding new biological insights and that may ultimately lead to innovative new medicines.

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