National Human Genome Research Institute (NHGRI)

Patented & Patent-pending Technologies Available for Licensing

Use of Farnesyl Transferase Inhibitors (FTIs) for Treatment of Laminopathies, Cellular Aging, and Arteriosclerosis

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Summary

Hutchinson-Gilford progeria syndrome (HGPS) is a genetic premature aging disease; affected children die of myocardial infarction or cerebrovascular accident at an average age of thirteen years. It is caused by a *de novo* single mutation in the LMNA gene, which encodes a component of nuclear lamina – lamin A/C. Thus, HGPS is a laminopathy. This mutation causes a deletion in lamin A and results in the accumulation of a truncated protein, called progerin. Progerin is improperly farnesylated and, in turn, the mutated protein contributes to abnormal nuclear scaffolding. NHGRI researchers have discovered that farnesyl transferase inhibitors (FTIs), both direct effectors and indirect inhibitors, can reduce the formation of abnormal nuclear morphology. Therefore FTIs have the potential to be used to as a therapeutic drug to treat HGPS patients as well as those diagnosed with other laminopathy disorders.

Potential Commercial Applications

Preclinical and clinical testing of FTIs as candidate drugs to treat HGPS and other laminopathies, as well as related conditions, such as arteriosclerosis (e.g., atherosclerosis), osteoporosis, bone deformations, and cellular aging.

Related Articles

Cao, K. et al., Research Article, *Progerin and Telomere Dysfunction Collaborate to Trigger Cellular Senescence in Normal Fibroblasts*, 121 J. CLIN. INVEST. 2833 (2011). *http://www.jci.org/articles/view/43578/pdf*

Collins, F. et al., *Inhibiting Farnesylation of Progerin Prevents the Characteristic Nuclear Blebbing of Hutchinson-Gilford Progeria Syndrome*, 102 PROC. NAT'L ACAD. SCI. OF THE U.S.A. 12879 (2005). *http://www.pnas.org/content/102/36/12879.full.pdf+html*

Key Words

Farnesyl Transferase Inhibitors, Hutchinson-Gilford Progeria Syndrome, Arteriosclerosis, Aging, Lamin A, LMNA Gene, Laminopathy