Vector For Gene Silencing And Replacement And Methods Of Use Thereof

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The Invention

An expression cassette for gene silencing and replacement, including, in operable communication, a promoter, an expression attenuator, a nucleotide sequence encoding a gene for a replacement target protein, and an shRNA sequence for knockdown of an endogenous variant of the target protein, wherein the promoter, the expression attenuator, the nucleotide sequence encoding the gene for the replacement target protein, and the shRNA are expressed as a single transcript. Also included are expression vectors and cells. Also included are methods of silencing and replacement of a target gene in a cell in culture by transforming the cells with the expression vectors described herein. Also included are minimal expression cassettes suitable for therapeutic methods.

Additional Information

For More Information About the Inventors

- Erik Dent

Tech Fields

- Drug Delivery : Other drug delivery technologies
- Research Tools : DNA & RNA tools

For current licensing status, please contact Jennifer Gottwald at jennifer@warf.org or 608-960-9854

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