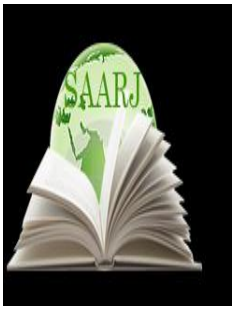

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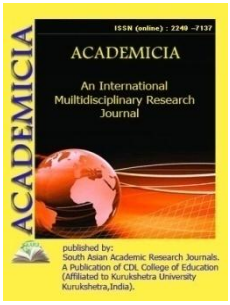

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CURING GENETIC DISEASE WITH GENE THERAPY: A REVIEW

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ABSTRACT

Development of viral vectors that enable high efficiency gene transfer into mammalian cells in the early 1980s anticipated the treatment of severe monogenic illnesses in humans. The use of gene transfer utilizing viral vectors has proven effective in disorders of the blood and immune systems, although with many curative trials also revealing severe adverse effects (SAEs) (SAEs). In children with X-linked severe combined immunodeficiency (SCID-X1), chronic granulomatous disease, and Wiskott-Aldrich syndrome, these SAEs were induced by incorrect activation of oncogenes. Subsequent investigations have identified the vector sequences responsible for these changing processes. Members of the Transatlantic Gene Therapy Consortium [TAGTC] have jointly created novel vectors that have proved safer in preclinical tests and utilized these vectors in new clinical trials in SCID-X1. These studies have showed indications of early effectiveness and preliminary integration analysis results from the SCID-X1 trial indicate a better safety profile.

KEYWORDS: *Diseases, Gene, Genetic, Therapy, Vectors.*

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