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(54) Title: THERAPEUTIC INTERVENTION IN A GENETIC DISEASE IN AN INDIVIDUAL BY MODIFYING EXPRESSION OF AN ABERRANTLY OR ABNORMALLY EXPRESSED GENE

(57) Abstract: The present invention provides means and methods for alleviating genetic disease. A genetic defect that has a phenotype in differentiated cells can lead to defects in precursor cells thereof. These so-called secondary defects contribute to the overall disease of the individual. In the present invention, genetic intervention with the aim to alleviate symptoms of genetic disease is directed toward the primary genetic defect in the differentiated cell and the secondary defect in the precursor cell.