

METHODS FOR INCREASING DNA COPY NUMBER

INNOVATION AND DESCRIPTION OF THE TECHNOLOGY

Abnormal mitochondrial signatures and mitochondrial dysfunction are increasingly associated with human disease. Alterations of mitochondrial DNA (mtDNA) copy number have been associated with a wide variety of phenotypes and diseases. Low copy numbers of mtDNA are associated as mitochondrial DNA depletion syndromes (MDS) as well with primary mitochondrial DNA disorders such as Lebers Disease (LHON), MELAS, Leigh Syndrome and Complex I deficiency.

There remains a need for treatment of diseases and disorders characterized by abnormal mitochondrial signatures and dysfunction, such as low mDNA copy number (MTCN).

The present invention disclosure Methods for increasing the mtDNA copy number in cells and tissue are provided, as well as methods for treating disease and disorders associated with a reduced mtDNA copy number.

MARKET AND ADVANTAGES OF THE TECHNOLOGY

The methods disclosed have the advantage of increasing mDNA copy number (MTCN) in a cell or tissue. Furthermore, these methods are useful for treating diseases or disorders associated with low MTCN, such as mitochondrial DNA depletion and deletions syndromes (MDDS) and primary mitochondrial disorders.

The methods of the present invention have the advantage of providing an effective amount of deoxythymidine or a prodrug in the absence of any other deoxyribonucleoside.

IPRS AND CONTACT

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