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Viewpoint

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Compositions and Methods for the Treatment of Mitochondrial Diseases

Patent Highlight

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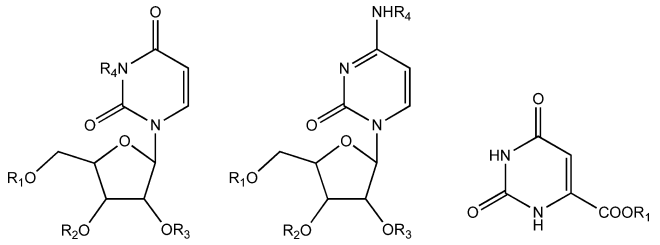
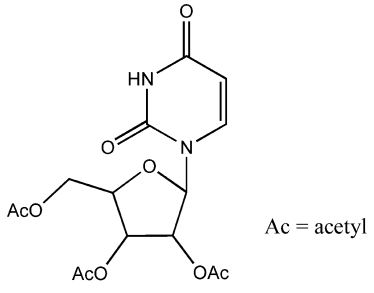
Title:	Compositions and Methods for the Treatment of Mitochondrial Diseases
Patent/Patent Application Number:	US 7915233 B1 **Publication Date:** March 29, 2011
Inventors:	Von Borstel, R. W.
Assignee Company:	Wellstat Therapeutics Corporation, USA
Disease Area:	Disorders related to mitochondrial dysfunction
Summary:	This application claims a series of pyrimidine nucleotide precursors as potential treatment for a wide variety of disorders caused by mitochondrial dysfunction. The invention claims are (i) a method for treating neurodegenerative diseases such as Alzheimer's (AD) and Huntington's disease (HD) and (ii) a method for treating congenital mitochondrial diseases selected from the group consisting of mitochondrial encephalomyopathy, lactic acidemia, Lerber's hereditary optic neuropathy, mitochondrial neurogastrointestinal encephalomyopathy, progressive external ophthalmoplegia, and Leigh's disease.
Important Compound Classes:	
Key Structures:	 Ac = acetyl Triacetoxyuridine
Biological Assay:	Triacyluridine is administered to patients suffering of multisystem mitochondrial disorder, refractory epilepsy, renal tubular acidosis, or developmental delay. In addition, triacyluridine is evaluated in the MPTP model of Parkinson's disease and mitochondrial dysfuntion and in the 3-nitropropionic acid model of HD.
Biological Data:	Triacyluridine improved patients' condition and showed efficacy in the in vivo models described.
Additional Information:	Mitochondrial disease refers to disorders to which deficits in mitochondrial respiratory chain activity contribute in the development of pathophysiology of such disorders in a mammal.

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Notes

The authors declare no competing financial interest.

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