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# Compositions and Methods for the Treatment of Mitochondrial Diseases

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# Compositions and Methods for the Treatment of Mitochondrial Diseases

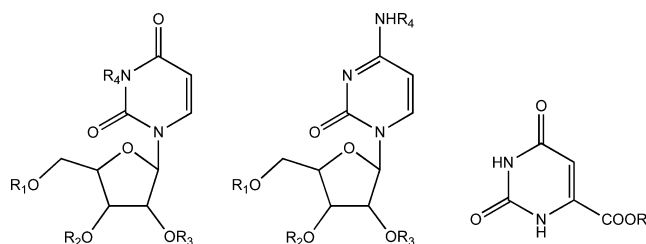
## Patent Highlight

Gerard Rosse\*

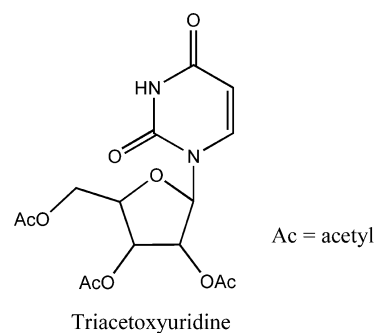
Structure Guided Chemistry, Dart Neuroscience LLC, 7473 Lusk Boulevard, San Diego, California 92121

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:



Biological Assay:	Triacetyluridine is administered to patients suffering of multisystem mitochondrial disorder, refractory epilepsy, renal tubular acidosis, or developmental delay. In addition, triacetyluridine is evaluated in the MPTP model of Parkinson's disease and mitochondrial dysfunction and in the 3-nitropropionic acid model of HD.
Biological Data:	Triacetyluridine 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.

## AUTHOR INFORMATION

### Corresponding Author

\*E-mail: grosse@dartneuroscience.com.

### Notes

The authors declare no competing financial interest.

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