

**Technology**

Use of the histone deacetylase inhibitor, sodium butyrate for Huntington's disease therapy

**Inventor**

Robert Ferrante, Ph.D.  
Bedford VA Medical Center  
Bedford, MA

**Key Features**

- Demonstrated to increase neuroprotection and improve motor performance
- Could be used either alone or in combination with other agents
- Expected to have mild or no toxicity

**Stage of Development**

Reduced to practice with successful demonstration in both in-vitro and animal models

**Keywords**

Therapeutic

- Huntington's disease
- CNS
- Neurodegeneration

**Patent Status**

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**Contact**

Ken Levin, Ph.D.  
Technology Transfer Program  
Department of Veterans Affairs  
Office of Research & Development (12TT)  
810 Vermont Avenue, NW  
Washington, DC 20420  
Phone: 202-461-1713  
Fax: 202-254-0460  
E-mail: [Ken.levin@va.gov](mailto:Ken.levin@va.gov)

## Use of the Histone Deacetylase Inhibitor, Sodium Butyrate for Huntington's Disease Therapy (VA Reference No. 03-111)

*Novel use of sodium butyrate to promote neuroprotection, improve motor performance, and reduce weight loss in Huntington's disease*

**Technology**

The Department of Veterans Affairs' novel technology involves the use of the histone deacetylase inhibitor, sodium butyrate to promote neuroprotection, improve motor performance, and reduce weight loss in Huntington's disease.

**Description**

It is possible that interference with gene transcription leading to disruption of normal gene expression could be critical in therapeutic development for Huntington's disease. The VA has identified that the use of sodium butyrate, an HDAC inhibitor, significantly enhances survival and reduces neuropathological effects, as well as motor deficits in Huntington's disease animal models. Gene transcription is regulated by complex interactions between proteins and histones and by the modification of these molecules via acetylation, methylation and phosphorylation. Mutant huntingtin (HTT or Huntington Disease gene) reduces histone acetylation by binding to histone acetyltransferase and can thereby affect gene transcription. Drugs that prevent histone deacetylation, known as histone deacetylase (HDAC) inhibitors, can restore transcription in the presence of mutant huntingtin.

**Competitive Advantage**

Although various drugs are available for the treatment of Huntington's disease and its complications, there is no specific curative treatment.

This invention:

- Has been studied extensively in animals and is expected to have a good safety profile in humans.
- Could be used in therapy either alone or in combination with other agents.
- Has been demonstrated to increase neuroprotection, to improve motor performance, to improve gross brain weight and atrophy, and to improve striatal neuron atrophy in animal models.

**Status**

The Department of Veterans Affairs is looking for a partner for further development and commercialization of this technology through a license, and the VA inventors are available to collaborate with interested companies through a Cooperative Research and Development Agreement (CRADA).