

## Product

Formate Therapeutic

## Indication

Homocystinuria

## Value Propositions

- ▶ Effective on a normal protein diet
- ▶ Decreases homocysteine by 50% alone and >90% w/ combination therapy

## Market

- ▶ \$50 Million—Global HCU Therapeutic Market Size Annually

## Intellectual Property

- ▶ Patent pending\*
- ▶ Available for licensing

## Contact

Heather Callahan  
heather.callahan@cuanschutz.edu

Ref# CU4975H

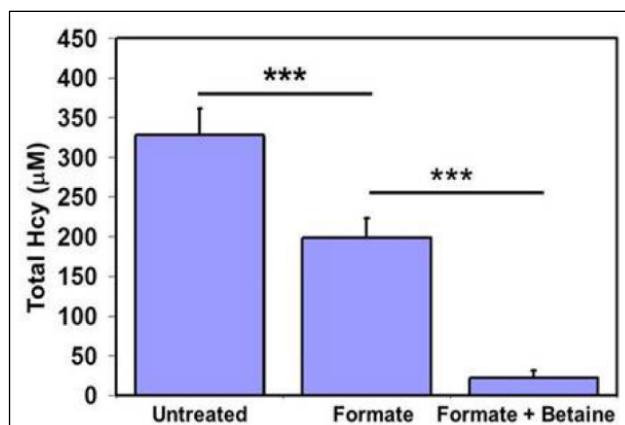
303-724-0220  
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## Background on CU4975H

Homocystinuria (HCU) is a rare metabolic condition characterized by an excess of homocysteine in the serum and urine caused by reduced activity of cystathionine beta-synthase (CBS). The disease is characterized by intellectual disability, hypercoagulability, nearsightedness, and displaced lenses of the eye if homocysteine levels cannot be kept below a certain level. Current treatments attempt to lower plasma homocysteine levels by restricting dietary intake of methionine and supplementation with betaine. However, dietary restriction often proves difficult to maintain, which results in poor outcomes for patients. Novel treatments could reduce the dependence upon methionine restriction.

## Technical Innovation

A University of Colorado team led by Dr. MacLean has shown that a formate-betaine combination treatment dramatically decreases homocysteine levels in HCU and removes the need for a methionine restricted diet in mice. In vivo treatment of HCU mice with sodium formate halved plasma homocysteine levels. The formate-betaine combination treatment resulted in the lowest levels of homocysteine ever observed in an HCU animal model to date. Such dramatic reduction in homocysteine levels in the setting of a methionine unrestricted diet may mean that individuals with HCU could resume a more normalized diet while on therapy. The inventors envision that the combination treatment could become the standard of care for all HCU individuals.



**Figure:** Formate and formate-betaine combination treatment near normalized plasma homocysteine levels in a HCU mouse model on a normal methionine diet.

### Resources & Documents:

\* US, EU, CA, and AU patents pending: "Compositions And Methods For Treating Homocystinuria And Other Conditions"